

# In the United States Court of Federal Claims

## OFFICE OF SPECIAL MASTERS

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TODD SIMANSKI and JULIA SIMANSKI, \*  
as Parents and Next Friends of \*  
O.A.S, a minor \*

Petitioners,

v.

SECRETARY OF HEALTH \*  
AND HUMAN SERVICES, \*

Respondent. \*

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No. 03-103V  
Special Master Christian J. Moran  
Filed: August 20, 2013  
Reissued: January 16, 2014  
Entitlement, disease disputed,  
GBS or CIDP versus SMARD

Ronald C. Homer and Sylvia Chin-Caplan, Conway, Homer & Chin-Caplan, P.C.,  
Boston, MA, for Petitioners;  
Traci R. Patton and Debra Filteau Begley, United States Dep't of Justice,  
Washington, DC, for Respondent.

### **PUBLISHED DECISION DENYING COMPENSATION<sup>1</sup>**

Todd and Julia Simanski alleged various vaccines caused their daughter, O.A.S., to suffer a disease of the peripheral nervous system, either Guillain-Barré Syndrome (“GBS”) or chronic inflammatory demyelinating polyneuropathy (“CIDP”). The Simanskis sought compensation pursuant to the National

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<sup>1</sup> This Decision was originally filed on August 20, 2013. On September 3, 2013, petitioners moved for redactions. Thereafter, on January 16, 2014, the undersigned granted petitioners’ motion for redaction. In this reissued version, the minor child’s name is redacted to initials, the minor’s birthdate is omitted, and this footnote is changed to reflect the redaction. The remainder of the Decision is unchanged.

Childhood Vaccine Injury Compensation Program, codified at 42 U.S.C. § 300aa—10 et seq. (2012).

When O.A.S. was two months old, she received a set of vaccines. Four days later, she suffered an episode of respiratory arrest and was hospitalized. The doctors diagnosed her as having a problem in her peripheral nerves. In 2001, shortly after O.A.S.'s vaccinations, GBS was considered the probable diagnosis by at least one treating physician. From 2003 on, however, O.A.S.'s treating doctors consistently diagnosed her as having spinal muscular atrophy with respiratory distress ("SMARD"), and not GBS.

Pursuant to instructions from the Federal Circuit, a hearing was held on February 4-7, 2013, with a fifth day of testimony on February 20, 2013. The Simanskis called two experts, Dr. Yehuda Shoenfeld (an immunologist) and Dr. Paul Maertens (a pediatric neurologist). The Secretary also called two experts, Dr. Christine McCusker (a pediatric immunologist) and Dr. Richard Finkel (a pediatric neurologist).

As outlined in their expert reports and restated in the oral testimony, the parties' respective experts came to markedly different positions regarding O.A.S.'s case. Dr. Maertens maintained that a set of vaccinations given to O.A.S. at age two months caused her to suffer from either GBS or CIDP.<sup>2</sup> To this foundation, the Simanskis add the opinion of Dr. Shoenfeld, who stated that the vaccines caused O.A.S.'s neurologic problem.

The Secretary challenged both propositions. First, primarily based upon the opinion of Dr. Finkel, the Secretary asserted that O.A.S. did not suffer from either GBS or CIDP. Instead, O.A.S. suffers from a different neurologic problem, SMARD. SMARD is caused by a genetic mutation. Second, even assuming that O.A.S. suffered from GBS or CIDP, the Secretary contended that the Simanskis have not met their burden of establishing that the vaccines caused O.A.S.'s neurologic problem. This attempted refutation of Dr. Shoenfeld's opinion rests primarily upon Dr. McCusker's opinion.

The debate between Dr. Shoenfeld and Dr. McCusker is largely academic because the evidence overwhelmingly favors a finding that O.A.S. suffers from SMARD. After the genetic basis for SMARD started becoming well known to

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<sup>2</sup> Whether GBS should be distinguished from CIDP for diagnostic purposes is addressed in section II(A)(2).

pediatric neurologists in 2003, O.A.S.'s treating doctors have consistently stated that she suffers from SMARD. Dr. Finkel's retrospective evaluation of various tests performed on O.A.S. throughout her life supports the conclusion that O.A.S. suffers from SMARD and never suffered from GBS or CIDP. Since the Simanskis have failed to establish a predicate for Dr. Shoenfeld's opinion, it is not necessary to evaluate whether his opinion that vaccines can cause GBS or CIDP is persuasive.

For these reasons, the Simanskis are not entitled to compensation. A complete explanation follows.

## **I. Procedural History**

The Simanskis filed their petition in 2003. The Simanskis filed a report from Dr. Maertens, exhibit 11, and two reports from Dr. Shoenfeld, exhibit 16 and exhibit 18. These reports were deemed insufficient and the case was dismissed in 2010. 96 Fed. Cl. 588 (2010) (denying petitioners' motion for review regarding the special master's decision to dismiss the case). However, the Federal Circuit vacated that dismissal. The Federal Circuit's opinion contains a lengthy recitation of the "complex" procedural history of the case through that time. 671 F.3d 1368, 1370-78 (Fed. Cir. 2012).

After the Federal Circuit remanded the case to the Court of Federal Claims, the case was further remanded to the undersigned. Although the Court's May 9, 2012 order required another decision by October 31, 2012, the Court subsequently granted a joint motion for additional time. The order, filed August 9, 2012, extended the deadline for the decision for approximately one year, until August 31, 2013.

The Court's extension of time allowed the parties time to develop their cases. The initial task was for the Simanskis to file updated medical records. They did so on June 20, 2012, and on June 26, 2012.

Next, the Secretary submitted her report pursuant to Vaccine Rule 4. The Secretary observed that although the original petition, filed on January 17, 2003, referred to an "adverse reaction," the Simanskis had not filed an amended petition clarifying the injury for which they seek compensation, despite orders to file an amended petition. Resp't Rep't at 6, citing orders dated March 19, 2004 and August 6, 2004. The Secretary noted that Dr. Maertens and Dr. Shoenfeld "assume that [O.A.S.] suffers GBS or CIDP, whereas the most recent medical records

indicate that [O.A.S.] carries a diagnosis of spinal muscular atrophy.” Resp’t Rep’t at 6. The Secretary argued that the Simanskis had not established that O.A.S. suffers from GBS or CIDP. Id. at 11. The Secretary also asserted that even if O.A.S. suffered from either GBS or CIDP, then the Simanskis were still not entitled to compensation because the evidence purporting to show a causal connection between O.A.S.’s vaccination and her development of either of those diseases was not persuasive. Id. at 11-12.

As to the immunologic theories Dr. Shoenfeld presented in his reports, the Secretary filed a report from Dr. McCusker, Resp’t Exhibit B. The Simanskis filed a supplemental report from Dr. Shoenfeld, exhibit 24. Dr. McCusker responded in another report, exhibit W.

The immunologists’ exchange was mirrored by that of the neurologists. Since the Simanskis had filed Dr. Maertens’s medical expert report (exhibit 11) before the Federal Circuit’s remand, the Secretary was obligated to respond. The Secretary responded by submitting Dr. Finkel’s first report, exhibit N, on September 28, 2012.<sup>3</sup> Dr. Finkel opined that O.A.S. suffered from SMARD, not GBS or CIDP. Exhibit GG.

The Simanskis filed a response from Dr. Maertens. He challenged the diagnosis of SMARD. Exhibit 37. He provided, however, relatively little affirmative information as to why his alternative diagnoses (GBS and/or CIDP) were correct. To remedy this omission, the Simanskis filed another report from Dr. Maertens, exhibit 41. Here, Dr. Maertens discussed more specifically why GBS and/or CIDP was the correct diagnosis.<sup>4</sup>

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<sup>3</sup> Dr. Finkel later amended his report by adding citations to O.A.S.’s medical records. The Secretary filed this amended report as exhibit GG. This decision cites to exhibit GG.

<sup>4</sup> The Simanskis revealed on December 28, 2012, the date they filed Dr. Maertens’s report, that they had provided him with a videotape of O.A.S. before her vaccinations. This disclosure prompted the Secretary to request all videotapes showing O.A.S. from her birth to January 26, 2001. (Later, the Secretary narrowed the request to videotapes from January 1, 2001 to January 26, 2001.) The Simanskis stated that the video clip that they sent to Dr. Maertens was the only videotape of O.A.S. they had for the relevant time. Exhibit 45.

The final neurologist's report was written by Dr. Finkel and filed as exhibit HH. In conjunction with this report, the Secretary presented Trial Exhibit A, a PowerPoint presentation regarding SMARD and GBS/CIDP.<sup>5</sup>

In addition to all these reports and the medical articles cited therein, the parties also filed briefs before the hearing. These briefs reflect different points of emphasis. The Simanskis' brief spends approximately 20 pages summarizing O.A.S.'s history for her first 15 months (birth in late-2000, through February 2002). The Simanskis' brief discusses various medical records after October 2003, but omits any discussion of the medical records mentioning that O.A.S. has SMARD. See Pet'r Prehear'g Br., filed Dec. 21, 2012, at 23-26. In contrast, the Secretary's brief states "[O.A.S.] has consistently carried this diagnosis [SMARD] from late 2003 to the present" and cites medical records supporting that statement. See Resp't Prehear'g Br., filed Jan. 15, 2013, at 3-4.

This difference between the two briefs was consistent with the difference in the opinions presented by Dr. Maertens and Dr. Finkel. Dr. Maertens's reports largely ignore the post-2003 statements of O.A.S.'s doctors that she had SMARD. Dr. Finkel, however, shares their opinions that O.A.S. suffers from SMARD.

The pre-trial briefs make clear that the dispute over the correct diagnosis is a critical issue in this case. Through Dr. Shoenfeld, the Simanskis maintain that the January 26, 2001 vaccinations caused O.A.S. to develop GBS/CIDP. The Simanskis have not presented an alternative claim based upon SMARD. For example, the Simanskis have not argued – nor have they presented any meaningful evidence – that the vaccinations caused O.A.S. to suffer SMARD.

To comply with the Federal Circuit's decision, a hearing was held to receive testimony from the four experts. The witnesses' schedules made it easier to have the two immunologists, Dr. Shoenfeld and Dr. McCusker, testify during the first two days. The two neurologists testified during the second two days and again on February 20, 2013.

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<sup>5</sup> Respondent re-filed Dr. Finkel's PowerPoint presentation because slide 8 contained a blue line that was not present in the original exhibit. See Resp't Notice of Filing, filed Feb. 27, 2013; Resp't Trial Exhibit B.

Following the hearing, the parties filed their post-hearing briefs. See Pet’r Posthear’g Br., filed May 6, 2013; Resp’t Posthear’g Br., filed June 18, 2013; Pet’r Posthr’g Reply, filed July 22, 2013. The post-hearing briefs are similar to the pre-hearing briefs. The Simanskis focused on GBS as the proper diagnosis for O.A.S.,<sup>6</sup> and the Secretary maintained that O.A.S. suffers from the genetic condition, SMARD. Thus, the matter is ready for adjudication.

## **II. Facts**

Unfortunately, except for the very first two months of her life, O.A.S. has had extensive medical problems. The doctors treating O.A.S. have generated voluminous materials about her, as have the therapists who have cared for her. Much of this material is not relevant to determining whether a vaccine caused O.A.S.’s problems. The parties and their experts rely on only a few documents, which are discussed in detail below.

### **A. Nervous System and Diseases Affecting It**

#### **1. Structure of the Nervous System**

The nervous system connects the brain and other parts of the body. When the brain wants a part of the body to move, the brain generates an electrical impulse. This impulse follows a path that contains three parts. It starts in the brain, and travels down the spinal cord along an upper motor neuron. Transcript (“Tr.”) 587, 914-15. (The brain and the spinal cord comprise the central nervous system, which is the first part of the neuro-muscular system.) Within the spinal cord, the electrical impulse meets an anterior horn cell. The term “anterior horn cell” refers to the anatomic location of this cell. Tr. 912, 915. While one part of the anterior horn cell is located in the spinal cord, the axon extends outside of the spinal cord and links to the muscle at the neuromuscular junction. Tr. 911-12; Resp’t Tr. Ex. B at 4; see also Dorland’s Illustrated Medical Dictionary 1251 (32nd ed. 2012) (“Dorland’s”) (depicting motor neurons and peripheral nerve fibers). The portion of the nerve that is outside of the spinal cord is known as the

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<sup>6</sup> Although the Simanskis focused their arguments on GBS (rather than CIDP), their brief also referenced CIDP. Pet’r Posthr’g Br. at 22 (“[O.A.S.]’s EMG test results further support a diagnosis of GBS/CIDP.”). Thus, this decision discusses both GBS and CIDP.

peripheral nervous system, which is the second of three parts of the neuro-muscular system. The final part is the musculature.

When neurologists see a patient with a problem in his or her neuro-muscular system, like O.A.S., they attempt to locate the area with dysfunction within either the central nervous system, the peripheral nerves or the muscles. Tr. 909 (Dr. Finkel). Dr. Maertens made a similar point, analogizing the neuro-muscular system to plumbing. Tr. 703-05. O.A.S.'s treating doctors determined that her central nervous system and her muscles were not the cause of her problem. Thus, they isolated her problem to the peripheral nervous system.

Nerves that extend from the spinal cord can be classified as either a motor nerve or a sensory nerve. See Dorland's at 1267-68; see also Resp't Trial Exhibit at 9 (schematic depicting different types of nerves); Tr. 922-25 (discussing this slide). In a highly simplified way, a peripheral nerve cell (also called a neuron) consists of a cell body from which an axon extends. Dorland's at 1267. The axon transmits electrical impulses to another part of the body and is analogous to a wire. Larger axons are wrapped in myelin. Dorland's at 186-87. Myelin is the "substance of the cell membrane . . . that coils to form the myelin sheath. . . . [Myelin] serves as an electrical insulator." Dorland's at 1218.

The distinction between axon and myelin is important because one way to distinguish the diseases implicated in this case (GBS, CIDP, and SMARD) is to determine what part of the peripheral nerve is damaged. The different characteristics of GBS, CIDP and SMARD are described in the following sections. This summary will provide context for O.A.S.'s history, which is described below in section II.B.

## **2. GBS & CIDP**

Both GBS and CIDP are neuromuscular diseases involving sensory and motor nerves of the peripheral nervous system. Tr. 698, 877. The basic definitions of these conditions point to similarities and differences between them. GBS is a "rapidly progressive ascending motor neuron paralysis of unknown etiology, frequently seen after an enteric or respiratory infection." Dorland's at 1832. CIDP is a "slowly progressive, autoimmune type of demyelinating polyneuropathy characterized by progressive weakness and impaired sensory function in the limbs . . . usually with elevated protein in the cerebrospinal fluid. It . . . is related to Guillain-Barré syndrome." Dorland's at 1491.

With regard to pathogenesis, GBS and CIDP seem to share a common pathway. GBS is most commonly a demyelinating condition.<sup>7</sup> Tr. 585. Chronic inflammatory demyelinating polyneuropathy, as its name implies, is also a demyelinating condition.<sup>8</sup> Dorland's at 1491. Demyelination is the “destruction, removal, or loss of the myelin sheath of a nerve or nerves.” Dorland's at 486. Demyelination occurs segmentally, that is, only part of the nerve is affected. One of Dr. Finkel's slides graphically depicts a simplified example of segmental demyelination. Resp't Trial Exhibit A at 10.<sup>9</sup> When the myelin (insulation) is damaged, the conduction of the electrical pulse down the axon (wire) is impaired. Tr. 681.

A nerve conduction study will detect the loss of function. Specifically, a nerve conduction study will show a difference in the velocity of the electrical impulse along the nerve. In the proximal part of the nerve (meaning the portion that is closer to the brain), the velocity will be normal. However, below the location of demyelination, the velocity will slow. The different velocities indicate that demyelination has blocked the electrical impulse. Tr. 1085-86.

Doctors generally believe that the substance that attacks the myelin is part of the person's immune system. Dorland's definition for GBS states “[a]n autoimmune mechanism following viral infection has been postulated.” Dorland's at 1832. Whether the immune system of newborns is sufficiently strong to damage myelin was a disputed point between the two immunologists, Dr. Shoenfeld and Dr. McCusker. E.g. compare Tr. 269-78 (Dr. Shoenfeld) with Tr. 345-53, 383-85 (Dr. McCusker).

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<sup>7</sup> To be precise, neurologists recognize four sub-types of GBS. Tr. 1103-04; see also Dorland's at 1832. The most common form is a demyelinating type. Two of the subtypes involve the axon of the nerve, Acute Motor Axonal Neuropathy and Acute Motor-Sensory Axonal Neuropathy. Dorland's at 1268. However, these rare conditions occur almost exclusively in China, Tr. 1104, and there is no suggestion that O.A.S. suffered from either of those. The fourth subtype of GBS is known as the Miller-Fisher variant and there is also no contention that O.A.S. suffers from Miller-Fisher syndrome. Consequently, references to Guillain-Barré syndrome mean the most common (demyelinating) form.

<sup>8</sup> Unlike GBS, CIDP does not have an axonal form. Tr. 1226.

<sup>9</sup> The underlying source of this material is Boston Children's Hospital.

Dr. McCusker's view that a newborn's immune system is not robust enough to cause autoimmunity is in accord with the general incidence of autoimmune diseases, including GBS.<sup>10</sup> Very rare are reports of GBS in infants less than three months old. Tr. 710. There is some question about whether GBS can occur in a newborn at all. Tr. 715-16.

In most cases of GBS, the first nerves to be affected are the nerves that extend to the legs and feet. Tr. 895. According to Dorland's definition, GBS begins with "paresthesias of the feet." Dorland's at 1832. Dr. Maertens explained that "in the classic form of GBS you have an ascending presentation starting in the low extremities and going up, and obviously there are symptoms of weakness that seem to have been more significant in the low extremities than in the upper extremity." Tr. 700-01.

Accordingly, a common presenting symptom for GBS is difficulty walking.<sup>11</sup> Tr. 712, 894. Dr. Finkel testified that "about a third of children will have preceding pain," either foot pain or back pain, and then their walking becomes unsteady and they start falling. Tr. 893-94. Dr. Maertens explained that "most of the kids [who suffer from GBS] are already walking . . . so they are going to have what is called either ataxia or inability to walk" as a presenting symptom. Tr. 709.

Dr. Maertens stated that it is "extremely difficult to make a diagnosis of GBS in a very young infant . . . because the child doesn't walk, doesn't sit up, and it's a lot easier to miss. It's going to be the hardest thing to diagnose." Tr. 715-16. Dr. Finkel explained that "you try to get a sense in an infant or a young child, who

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<sup>10</sup> Some autoimmune diseases are called congenital because they originate with how the mother's immune system interacts with her fetus. Tr. 272-73. GBS is not considered a congenital autoimmune disease.

<sup>11</sup> Dr. Finkel carefully distinguished between "first symptom" and "presenting symptom." The presenting symptom is what prompts the person to go for medical treatment. The "presenting symptom" may actually be preceded by other symptoms, which were not severe enough to cause the person to seek medical care. Tr. 1211-12; see also Dorland's at 1817.

may not be able to articulate that well, are they having pain. And it might be foot pain or back pain, back pain if the nerve roots are inflamed.” Tr. 893.

After GBS starts, the disease usually progresses quickly. It typically begins with “paresthesias of the feet, followed by flaccid paralysis of the entire lower limbs, ascending to the trunk, upper limbs, and face; other characteristics include slight fever, bulbar palsy, absent or lessened tendon reflexes, and increased protein in the cerebrospinal fluid.” Dorland’s at 1832. As more proximal nerves are affected, the person may lose the ability to breathe due to impairment in the phrenic nerve. (The phrenic nerve enervates the diaphragm, which is the muscle that moves the lungs in respiration. Tr. 681.) Difficulty breathing, though, is rarely a presenting symptom for GBS. Tr. 711.

The Asbury criteria establish the symptoms frequently used to diagnose GBS. Tr. 728-29. The criteria include elevated protein in the spinal fluid and response to IVIG treatment. Tr. 717-18. Common symptoms also include “reduced or absent reflexes.” Tr. 895. Dr. Finkel explained that the “reflexes are typically lost early in GBS/CIDP. That’s a hallmark. That’s one of the two main criteria of GBS.” Tr. 1208. Dr. Maertens agreed with Dr. Finkel’s explanation, he stated that “[i]n most cases, the reflex[es] are decreased or lost.” Tr. 1229.

Typically in GBS, the destruction of myelin produces an increased amount of protein in the fluid that envelops the spinal cord. Tr. 713. Both Dr. Finkel and Dr. Maertens explained that for their patients in whom they suspect GBS, they order an MRI of the spine, followed by a spinal tap looking for the elevated protein. Tr. 713-14, 896-97.

Once a doctor suspects that a patient has GBS, it is important to treat with IVIG. Dr. Maertens asserted that “if you strongly believe that’s what it is, you cannot delay treatment.” Tr. 717. He also explained that “the response to treatment is going to help you in establishing your diagnosis too, because if you have a response . . . it’s a supportive finding.” Id. Dr. Finkel testified that “if we have a strong clinical suspicion and we’ve excluded other things along the way, then we start the IVIG. We move ahead with treatment.” Tr. 898.

By definition, GBS is an “acute” neuropathy. Dorland’s at 1832. Generally, “acute” means “having a short and relatively severe course.” Dorland’s at 24. Dr. Finkel testified that the maximal impairment for GBS is “typically within one to two weeks, as much as four weeks, and then you see recovery . . . back to baseline or nearly baseline.” Tr. 1121. People who suffer from GBS can completely

recover or have a nearly complete recovery. Tr. 778. Dr. Maertens opined that most cases of GBS resolve, but sometimes GBS can relapse. Tr. 619, 719-20. Dr. Finkel stated that GBS relapses about 15 percent of the time in children. Tr. 900.

The duration of GBS is a primary way of distinguishing it from chronic inflammatory demyelinating polyneuropathy. As the name implies, CIDP is a chronic condition, meaning it “persists over a long period of time.” Dorland’s at 359 (defining “chronic”). Unlike GBS, which resolves quickly, CIDP can be diagnosed only if the patient suffers symptoms for eight or more weeks. Tr. 630-31.

Another difference between GBS and the common presentation of CIDP concerns how the disease appears initially. People with GBS decline rapidly. In contrast, CIDP is usually “slowly progressive.” Dorland’s at 1832. The “[p]resenting symptoms often include tingling or numbness of the digits, weakness of the limbs, hyporeflexia or areflexia, fatigue, and abnormal sensations.” Dorland’s at 1491. Dr. Maertens testified that “in CIDP you could have cumulative damage and eventually lack of recovery.” Tr. 778.

A complication with CIDP is that not all instances of CIDP are “slowly progressive.” Some cases of CIDP present acutely. Tr. 1226. At the onset of problems, neurologists cannot tell if the acute presentation is the start of GBS or the start of CIDP. Because CIDP is, by definition, a “demyelinating” disease, results on EMGs and nerve conduction studies are similar. Tr. 980. Dr. Finkel opined that there should be evidence of segmental slowing in both GBS and CIDP. Id.

The sudden-onset CIDP cases present a diagnostic challenge. Dr. Maertens opined that there is a spectrum of inflammatory neuropathies, and that an acute presentation can evolve into a more chronic form. Tr. 629. Dr. Finkel stated that he does not think that GBS turns into CIDP, but acknowledged that “it may be a semantic issue.” Tr. 900. He explained that there is “a small group, small percentage of CIDP, that can present acutely, and it can look like GBS. So it is a demyelinating disease, just like GBS.” He stated that in these cases one might originally think the condition is GBS “because it has the features of it. It’s a demyelinating, acute onset neuropathy. But two months later, after you’ve had a chance to see how it has evolved, you have to rethink and say, okay, that was actually the initial presentation of CIDP.” Tr. 900-01.

Whether GBS and CIDP are separate and distinct clinical entities or they belong on a spectrum of similar diseases is a difficult question that has appeared periodically in cases in the Vaccine Program. E.g., *Tompkins v. Sec’y of Health & Human Servs.*, No.10-621V, 2013 WL 3498652, \*27 (Fed. Cl. Spec. Mstr. June 21, 2013), mot. for review filed (July 22, 2013); *Torday v. Sec’y of Health & Human Servs.*, No. 07-372V, 2009 WL 5196163 (Fed. Cl. Spec. Mstr. Dec. 10, 2009); *Kelley v. Sec’y of Health & Human Servs.*, 68 Fed. Cl. 84 (2005). A resolution of that question, however, is not required in this case because, for the reasons explained below, O.A.S. has not suffered from either GBS or CIDP. Rather, she suffers from an entirely different disease, SMARD.

### 3. SMARD

SMARD is “characterized by a sudden onset of respiratory distress within the first 13 months of life and initially distal and later generalized muscular weakness.” Exhibit P (Maria Eckart et al., *The Natural Course of Infantile Spinal Muscular Atrophy with Respiratory Distress Type 1 (SMARD1)*, 129 *Pediatrics* e148 (2012)) at e149. For SMARD, “[t]he clinical picture is characterized by initial respiratory insufficiency due to diaphragmatic palsy and often followed by distally pronounced weakness and wasting.” Exhibit V (S. Rudnik-Schöneborn et al., *Long-Term Observations of Patients with Infantile Spinal Muscular Atrophy with Respiratory Distress Type 1 (SMARD1)*, 35 *Neuropediatrics* 174 (2004)) at 174.

SMARD is not the same as a disease with a similar name, spinal muscle atrophy (“SMA”). According to Dr. Maertens, there is “[q]uite a significant difference” between those conditions. Tr. 654. Years ago, the term “spinal muscle atrophy” was a taxonomic category covering both conditions. However, doctors now distinguish them. Tr. 878-79, but see exhibit V (Rudnik-Schöneborn).

Most pediatric neurologists became aware of the distinction between SMA and SMARD in 2003, when the *Annals of Neurology* published an article on SMARD. Tr. 756-57, 882-83, 906-07; see also exhibit S (Katja Grohmann et al., *Infantile Spinal Muscular Atrophy with Respiratory Distress Type 1 (SMARD1)*, 54 *Ann. Neurol.* 719 (2003)). Dr. Maertens testified that he first learned about SMARD in 2005 or 2006. Tr. 803.

One reason for treating SMARD and SMA as different entities is that they have different origins. The basis for most cases of SMA is a genetic mutation, located on chromosome 5q, which was identified in 1995. Tr. 878. In contrast,

SMARD involves a different gene, known as IGHMBP2.<sup>12</sup> See exhibit P (Eckart) at e149; Tr. 831.

Another reason for differentiating between SMA and SMARD is that their presentations are “inverted.” Tr. 654 (Dr. Maertens). SMA “is more proximal and involves the big muscles around the hip girdle typically.” Tr. 879. In contrast, SMARD starts distally and often involves the diaphragm. Tr. 654.

Although SMA and SMARD are different diseases, both are considered diseases of the anterior horn cell.<sup>13</sup> Tr. 634, 651, 655, 913, 1075-76. For SMARD, “the important thing is that there is pathology along the nerve fiber and all the way distally at the end, at the terminal arborization where the nerve fiber has to connect to the muscle through the neuromuscular junction.” Tr. 912-13. In Dr. Finkel’s view, SMARD is a peripheral neuropathy “because it affects the axon and because it affects the terminal arborization of how the nerve is connecting to the muscle.” Tr. 913-14.

## **B. O.A.S.’s History**

A brief overview of O.A.S.’s history begins with her birth.<sup>14</sup> She did not weigh very much when she was born. During her first two months, however, she gained weight and appeared to be healthy. At two months, she received a set of vaccinations.

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<sup>12</sup> Some of the medical literature filed distinguishes between types or variants of SMARD. See e.g., exhibit P (Eckart) at e149. The parties’ experts, however, did not refer to any specific variant. Thus, this decision refers to SMARD generally.

<sup>13</sup> As explained in section II.A.1., the anterior horn cell is a transition point, corresponding to where the nerve leaves the central nervous system (the spinal cord) and enters the peripheral nervous system.

Other diseases of the anterior horn cell include polio and Lou Gehrig’s disease. Tr. 100 (Dr. Shoenfeld), 588 (Dr. Maertens), 913 (Dr. Finkel).

<sup>14</sup> See Simanski, 671 F.3d at 1370 (providing O.A.S.’s medical history in three paragraphs).

Four days after the vaccinations, she suffered respiratory arrest. She was hospitalized at a local institution, Mercy Medical Center, for nearly one month. From there, she was transferred to the Mayo Clinic, where she remained for approximately three weeks. She went back to Mercy, went home, and then returned to Mercy. After her third stay at Mercy, she went to Johns Hopkins University for approximately ten days. Next, she went to the University of Iowa, which was closer to her home, for more than three months. Throughout this time, O.A.S.'s respiratory problems persisted, although she improved for about one month.

In 2003, she returned to the Mayo Clinic and a doctor indicated that she may have SMARD. After 2003, her treating doctors have generally stated that O.A.S. suffers from SMARD. Their current diagnosis, SMARD, is arguably in conflict with diagnoses given in 2001, when O.A.S. first experienced respiratory distress.

To explain the basis for the various doctors' opinions about the diagnosis for O.A.S., a more detailed recitation about the test results and the doctor's interpretation of those test results follows. The subsequent chronology is basically organized by the institution caring for O.A.S. and focuses upon the medical records discussed by the testifying neurologists.<sup>15</sup>

### **1. Health through January 26, 2001, the Date of Vaccination**

Ms. Simanski's pregnancy with O.A.S. appeared uncomplicated. During her birth in late-2000, O.A.S. experienced fetal distress, requiring the doctors to perform a C-section. O.A.S.'s weight was low – only 2180 grams (4 pounds 12.9

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<sup>15</sup> As noted in the procedural history, each party retained immunologists. The testimony of Dr. Shoenfeld and Dr. McCusker has been considered. However, they have much less expertise in diagnosing neurological problems in infants. See Tr. 163-64 (Dr. Shoenfeld deferring to pediatric neurologists to discuss SMARD). Thus, the recitation of facts cites to the testimony of the treating neurologists.

ounces), and she was diagnosed with intrauterine growth retardation (“IUGR”).<sup>16</sup> Pet’r Exhibit A at 3 (discharge summary); Pet’r Exhibit B at 100, 102.<sup>17</sup>

In Dr. Finkel’s opinion, O.A.S.’s IUGR was “highly consistent” with, and supportive of, the diagnosis of SMARD. Exhibit GG at 2; Tr. 934. To buttress his opinion, Dr. Finkel cited a factor-analysis study that analyzed the different clinical features associated with SMARD. Tr. 1018-19 (discussing exhibit T (Ulf-Peter Guenther et al., Clinical and Mutational Profile in Spinal Muscular Atrophy with Respiratory Distress (SMARD): Defining Novel Phenotypes Through Hierarchical Cluster Analysis, 28 Hum. Mutation 808 (2007)) at 810, Table 1).

On the other hand, Dr. Maertens’s opinion regarding the significance of O.A.S.’s IUGR seemed to vary. In his report, he stated that he disagreed with Dr. Finkel’s opinion that IUGR was consistent with SMARD. Exhibit 37 at 1. However, during cross-examination, Dr. Maertens acknowledged that approximately three-quarters of the patients with SMARD also had IUGR. Tr. 766. Still later in his testimony, Dr. Maertens indicated that O.A.S.’s IUGR was not a useful piece of data in distinguishing whether she had GBS/CIDP or SMARD. Tr. 810-11.

After her birth, O.A.S. gained weight and moved up the growth charts. At two weeks and one month of age, Ms. Simanski brought O.A.S. to see her pediatrician, Dr. Emily Gavin. These two visits were relatively routine. Pet’r Exhibit C at 196-98. Dr. Maertens emphasized that O.A.S. did well during her first two months. Tr. 572. Dr. Gavin’s records do not note any problems with a failure to nurse. Pet’r Exhibit C at 197-98. Dr. Maertens plotted O.A.S.’s weight on a standard growth chart and determined that she was catching up to her peers.

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<sup>16</sup> Approximately five months after O.A.S. was born, Ms. Simanski told one of O.A.S.’s neurologists that her placenta was calcified and placental insufficiency was the presumed cause of O.A.S.’s small size at birth. Exhibit 5, Vol. 1, at 277.

<sup>17</sup> The Simanskis’ initial attorney of record filed a set of exhibits with letters. The usual practice in the Vaccine Program is for petitioners to use numbers. The Simanskis’ current attorney has followed this convention and numbered petitioners’ subsequently filed exhibits. Thus, any exhibit labeled with a number was filed by the Simanskis and any exhibit labeled with a letter was filed by the Secretary, unless otherwise noted as “Pet’r Exhibit.”

Although when she was born, she was below the zero percentile, O.A.S. reached the twenty-fifth percentile by two months. Exhibit 48 at 2; Tr. 572. This impressive weight gain showed Dr. Maertens that O.A.S. was healthy. Tr. 572-73. Dr. Finkel agreed that O.A.S. gained weight and that Dr. Gavin did not note any problems in early medical records. Tr. 1052, 1150.

Dr. Maertens and Dr. Finkel, though, depart in the significance of O.A.S.'s early good health. Dr. Maertens opined that her success was not consistent with developing SMARD. Tr. 655-60. Dr. Maertens explained that children who have an anterior horn cell disease, such as SMARD, have difficulty feeding and experience weight loss "because the diaphragm is very important for sucking." Tr. 655. Dr. Maertens testified that O.A.S. had "no sign of failure to thrive, which is a very common problem in any . . . spinal muscular atrophy kid." Tr. 660.<sup>18</sup>

Dr. Finkel, however, viewed O.A.S.'s early good health as consistent with SMARD. Dr. Finkel carefully distinguished SMARD and SMA because "[t]he oral motor muscles of feeding, the suck-swallow muscles, are not affected early in SMARD. So we don't see early feeding problems, as you would in typical SMA and other neuromuscular disorders. So these children typically don't need feeding tubes as early as SMA, for example." Tr. 1006.

On December 28, 2000, Mr. Simanski recorded a video of O.A.S. lying in a crib. She is wearing clothes that cover her feet, but her hands are exposed. In the video, Mr. Simanski can be heard interacting lovingly with O.A.S. O.A.S. makes some cooing sounds, typical for a newborn. O.A.S. also has one cry at around the three minute mark. In total, the video lasts approximately three minutes and twenty-two seconds. Exhibit 42.<sup>19</sup>

Dr. Maertens and Dr. Finkel had different opinions about O.A.S.'s cry. Dr. Maertens stated that the cry was a "normal cry." Tr. 1238. He explained that the cry was "[n]ot very loud, but she was not very mad. She cried like a baby who

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<sup>18</sup> Dr. Maertens discussed "spinal muscle atrophy" without specifying whether this term included SMARD. Tr. 660. Earlier, however, he said that both SMA and SMARD would cause problems in feeding. Tr. 656.

<sup>19</sup> Apparently, the Simanskis did not record (or retain) any other videos of O.A.S. in the next month. See footnote 4 above.

cries a little bit. They were not sticking her or doing something really mean, so she had no reason to be.” Tr. 666.

In contrast, Dr. Finkel testified that O.A.S.’s cry was “feeble” and “not a robust cry.” Tr. 933. Dr. Finkel stated, “[W]hen I looked at the video, it was my interpretation that her cry was weak. And I do understand the points that you’re making that the parents felt that she had a vigorous cry and a good suck, but I would note that she did not demonstrate a vigorous cry on the video.” Tr. 1149. If Dr. Finkel is correct, that O.A.S. had a weak cry, then this symptom would support the SMARD diagnosis because “a feeble cry can be the earliest symptom, the earliest feature, of SMARD, and it can precede the onset of respiratory failure.” Tr. 933.

On January 24, 2001, both Ms. Simanski and O.A.S. were having gastrointestinal problems (nausea, vomiting, increased spit up, diarrhea). Dr. Gavin diagnosed infectious gastroenteritis. Dr. Gavin deferred vaccinations due to O.A.S.’s ill health. Pet’r Exhibit C at 194.

O.A.S. received a set of vaccines on January 26, 2001. Specifically, Dr. Gavin’s office administered doses of the diphtheria-tetanus-acellular pertussis, hepatitis B, Haemophilus influenzae type B, inactivated polio, and pneumococcal vaccines. Id. at 141. These vaccines allegedly caused her neurological problems.

## **2. Mercy Medical Center – January 30, 2001 through February 23, 2001**

### **a) Summary<sup>20</sup>**

On January 30, 2001, O.A.S. had an episode of respiratory arrest. Her babysitter gave her rescue breaths and called for emergency assistance. An ambulance brought O.A.S. to the emergency room at Mercy Medical Center. Exhibit 15.

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<sup>20</sup> The discussion of each hospitalization begins with a brief summary. That summary is followed by a separate description of the most pertinent tests conducted on O.A.S. during that hospitalization. Dr. Maertens’s comments and Dr. Finkel’s comments concerning each important test are presented in this context.

To help with breathing, O.A.S. was intubated. In association with the intubation, O.A.S. was given a medication to sedate her to prevent her from removing the tube. Tr. 1190-91; see also Pet'r Exhibit D at 1277 (“She has been sedated off and on today for fighting the ventilators.”).<sup>21</sup> While in Mercy Medical Center, a lab test revealed the presence of human respiratory syncytial virus (“RSV”). Pet'r Exhibit D at 1446.<sup>22</sup> O.A.S. was diagnosed with bronchiolitis. Id. at 1274-76.

O.A.S. stayed in Mercy Medical Center until February 23, 2001. During those twenty-four days, many doctors examined her and many tests were conducted. O.A.S. failed extubation twice, although she eventually extubated herself. On February 23, 2001, her attending intensivist at Mercy, Dr. Napa, transferred O.A.S. to the Mayo Clinic. Dr. Napa's transfer report, which is found at Pet'r Exhibit D at 1260-62, summarized the events at Mercy.

Dr. Maertens and Dr. Finkel discussed the following aspects of O.A.S.'s care at Mercy extensively. These are presented in chronological order.

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<sup>21</sup> In response to a leading question from the Simanskis' attorney, Dr. Maertens stated that while O.A.S. was intubated, the doctors gave her a drug to paralyze her. Tr. 576-77. The testimony concerning paralysis was an exaggeration because Mercy medical records state that O.A.S. could move her extremities. Pet'r Exhibit D at 1279.

<sup>22</sup> Three of the four experts testified that O.A.S. was infected with RSV. Tr. 402-05 (Dr. McCusker), 579 (Dr. Maertens), 1171 (Dr. Finkel). The only expert who found the presence of a positive DNA test for RSV taken from O.A.S.'s nose not to be sufficient evidence of an RSV infection was Dr. Shoenfeld. Tr. 144-45. Given that Dr. Shoenfeld is also the only one of the testifying experts who does not regularly treat infants, his opinion is not credited. Dr. Shoenfeld's unwillingness to accept the results of what appears to be a straightforward test is inexplicable. See Doe v. Sec'y of Health & Human Servs., 76 Fed. Cl. 328, 338 (2007) (ruling that special master was not arbitrary in finding petitioner's expert not credible when he “doggedly stuck to his assumption that petitioner suffered no symptoms of infection prior to vaccination, even though the medical records stating otherwise were brought to his attention”).

## **b) Respiratory Function, including RSV and Diaphragmatic Weakness**

The detection of RSV explains why O.A.S. suffered respiratory distress a few days earlier. The RSV infection caused congestion in her upper respiratory tract and once her airways became obstructed, she had a respiratory arrest. Tr. 574, 686. In conjunction with the doctors' efforts to keep O.A.S. alive, they intubated her. Tr. 576. At this time, the doctors did not note any problems with her diaphragm. Tr. 570, 629, 701, 1189.<sup>23</sup>

On February 8, 2001, a doctor ordered a fluoroscopy for O.A.S. A fluoroscopy shows the motion of an organ. Tr. 1007, see also Mosby's Manual of Diagnostic and Laboratory Tests 1030 (4th ed. 2010) ("Mosby's"). For O.A.S., "the right hemidiaphragm [was] elevated. However, both hemidiaphragms move in a normal fashion." Pet'r Exhibit D at 1494.<sup>24</sup> Diaphragmatic weakness is not a consequence of an RSV infection. Tr. 1189.

Elevation in the diaphragm means it is weak. Normally, the diaphragm should push down into the abdomen. When the muscle is weak, it rises. Tr. 679, 682. A related medical term is "eventration." See Dorland's at 655.

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<sup>23</sup> Dr. Shoenfeld expressed the opinion that O.A.S. first suffered paralysis of the phrenic nerve, which stimulates the diaphragm, then suffered respiratory distress as a consequence of that paralysis. Tr. 86, 142. This opinion is inconsistent with the opinion of Dr. Maertens, the Simanskis' other expert. In Dr. Maertens's view, O.A.S. had RSV and then later developed GBS/CIDP with phrenic nerve involvement. Tr. 571.

Dr. Shoenfeld's opinion depends upon finding that O.A.S.'s doctors, who were treating her respiratory problem, failed either to observe or to record that her diaphragm was impaired. This assumption is highly unlikely. See Tr. 679-80. Thus, Dr. Shoenfeld's opinion that O.A.S. first suffered neurological problem and then suffered respiratory distress is rejected in favor of Dr. Maertens's alternative opinion.

<sup>24</sup> In contrast to Dr. Shoenfeld's opinion about phrenic nerve paralysis, the fluoroscopy indicated no evidence of diaphragmatic paralysis. Pet'r Exhibit D at 1494; see also Tr. 1072.

On February 20, 2001, O.A.S. had a chest x-ray. The doctor reported “elevation of the right hemidiaphragm is again seen.” Pet’r Exhibit D at 1454.

In Dr. Finkel’s opinion, elevation of the diaphragm, especially the right side, is consistent with SMARD. Tr. 920-21. Relying on the article by a group of German researchers (exhibit V (Rudnik-Schöneborn)), Dr. Finkel stated that O.A.S.’s diaphragmatic palsy (or weakness) is typical for SMARD. Tr. 1020, 1034-35.

According to Dr. Maertens, diaphragmatic weakness is “simply not part of RSV.” Tr. 1189. To him, O.A.S.’s diaphragmatic weakness meant that the doctors “have to look for something else.” Dr. Maertens recognized that diaphragmatic weakness was “a fundamental aspect of considering that a child has SMARD,” although diaphragmatic weakness has causes other than SMARD. Id.

### c) Creatinine Level

When O.A.S. was admitted to Mercy Hospital, she had blood drawn. One of the tests measured the amount of creatinine in her blood. Creatinine is a protein stored in the muscle, and it is produced as part of muscle contraction. Tr. 1180-81. The kidneys excrete creatinine through the urine. Tr. 1181; Mosby’s 204-06. Dr. Maertens explained that because the kidneys excrete creatinine, an elevation in creatinine can indicate the existence of a renal disease. Tr. 790-91.

A low amount of creatinine, however, suggests “a decrease in the amount of muscle tissue” because less creatinine is being excreted into the blood. Tr. 1181; see also Mosby’s at 204-06. O.A.S.’s creatinine level on January 30, 2001 was 0.1 mg/dl and was marked as low. Pet’r Exhibit D at 1436. The normal value range for creatinine for O.A.S.’s test was 0.3-0.5 mg/dl.<sup>25</sup>

Dr. Finkel opined that low levels of creatinine are consistent with a diagnosis of SMARD because low creatinine indicates “decreased muscle bulk.” Tr. 1091-92. He noted in his report that a low level of creatinine is also consistent with CIDP, but inconsistent with GBS. Exhibit GG at 3. He maintained that the

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<sup>25</sup> The current edition of Mosby’s lists slightly different values, depending on the age of the person. For infants, the normal range is 0.2-0.4 mg/dl, and for newborns, the normal range is 0.3-1.2 mg/dl.

reduction in muscle mass comes after weeks and is not an immediate process. Tr. 1004-05; exhibit HH at 4. Dr. Finkel explained that a low creatinine level is “a more general reflection of muscle health and it’s supportive of an underlying muscle condition.” Tr. 1181-82.

During his testimony, Dr. Maertens raised two points about O.A.S.’s creatinine level. First, Dr. Maertens seemed to argue that O.A.S.’s creatinine levels “were not low, were not very low, and they were frequently normal.”<sup>26</sup> Tr. 783. Second, Dr. Maertens maintained that results of creatinine tests from other times should be considered as well. Tr. 815. In Dr. Maertens’s view, the different results on the creatinine tests meant that the January 30, 2001 data point should not be used in determining whether O.A.S. suffered from GBS/CIDP or SMARD. *Id.*

#### **d) Dr. Narawong’s Neurologic Evaluation**

After O.A.S. had been in the Mercy Medical Center for about one week, she was observed to have “arching of the back and stiffening of the extremities.” Pet’r Exhibit D at 1277. O.A.S. also had “staring episodes where she would stare into space and had to be stimulated with tactile stimulation for her to come out of the episode.” *Id.* Thus, a neurologist, Duanchai Narawong, was consulted on February 8, 2001.

As part of his history of present illness, Dr. Narawong noted that O.A.S. “has been sedated off and on today for fighting the ventilators.” *Id.* The two experts in this case differed in their views about the consequence of sedation. Dr. Maertens suggested that O.A.S.’s medication would prevent a thorough neurologic examination, Tr. 674, because the only thing a neurologist can evaluate in a sedated patient is “the pupils, [and] the corneal reflex.” Tr. 578. Dr. Finkel explained that neurologists can conduct an examination of sedated children more thoroughly than Dr. Maertens described. Tr. 1069. Dr. Finkel routinely examines

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<sup>26</sup> Dr. Maertens proposed that O.A.S.’s low creatinine level could be “slightly lower than normal” because she was excreting the creatinine in her urine. Tr. 788-90. Dr. Maertens acknowledged, however, that he did not “know what the urine output is on this child,” so he could not testify as to whether she had received a lot of fluid and was urinating a lot. Tr. 790. Furthermore, O.A.S.’s blood urea nitrogen (“BUN”) test showed that her kidneys were functioning properly. Pet’r Exhibit D at 1436.

sedated children and is able to assess “almost everything,” including the cranial nerve function, motor tone, reflexes, and sensory responses. Tr. 1069.

Regardless of what a neurologist could have done, both Dr. Maertens and Dr. Finkel found Dr. Narawong’s evaluation to be quite limited. Tr. 827, 1072-73. Dr. Finkel maintained that it was “a bit of an embarrassing neurologic exam that seems to show no abnormalities.” Tr. 1072-73. The neurologic portion of O.A.S.’s physical examination stated, in its entirety: “The motor examination shows that she moves all extremities well. The muscle tone is normal. Deep tendon reflexes are 2+ throughout.” Pet’r Exhibit D at 1277. Dr. Narawong also indicated that the staring episodes were probably not seizures, and ordered an EEG.

Dr. Maertens stated that he “cannot believe” the results of Dr. Narawong’s neurologic exam because a 2+ on deep tendon reflexes is a normal result. A 2+ on deep tendon reflexes “means there’s nothing wrong with this child’s peripheral nervous system,” which Dr. Maertens maintained was inconsistent with the results of O.A.S.’s nerve conduction tests. Tr. 826-27. Dr. Finkel concluded that the exam was “rather cursory” and that the results do not support a diagnosis of SMARD, GBS, or CIDP. Tr. 1190-91.

### **3. Mayo Clinic – February 23, 2001 through March 16, 2001**

#### **a) Summary**

On February 23, 2001, Dr. Napa transferred O.A.S. to the Mayo Clinic. Pet’r Exhibit D at 1263-65 (discharge report). While at the Mayo Clinic, O.A.S. underwent more tests. For purposes of this decision, the important tests include a muscle biopsy, two electromyographies, and a lumbar puncture. At least one doctor indicated that O.A.S. suffered from a demyelinating process in her peripheral nerves. Pet’r Exhibit E at 2151 (Dr. Kotagal).

Doctors ordered a four-day course of intravenous immunoglobulin (“IVIG”) from March 7 to March 10, 2001. Pet’r Exhibit C at 314-15. After the IVIG, O.A.S. improved sufficiently enough that she was taken off the ventilator by March 14, 2001. *Id.* at 314 (providing history of respiratory function through March 16, 2001). Dr. Gavin, however, was uncertain as to whether the IVIG helped O.A.S. *Id.* at 188. On March 16, 2001, the Mayo Clinic doctors transferred O.A.S. back to Mercy Medical Center. *Id.* at 313-16.

## b) Electromyography Overview

A basic explanation of an electromyography (“EMG”) is provided because O.A.S. had many EMGs and the testifying neurologists extensively discussed this series of tests.

An EMG is often conducted in association with another test formally known as an electroneurography. Electroneurographies are more commonly known as nerve conduction studies. One purpose of a nerve conduction study is to locate an injury to the peripheral nerve. Mosby’s at 581. The nerve conduction study is done by initiating an electrical signal at a proximal point and measuring the latency, or how long the electrical signal takes to reach a distal point. Id. at 581-82, 594. When two or more distal points are used, the nerve conduction study can measure distal velocity. Mosby’s at 581-82.

Dr. Maertens testified that, as a clinician, he sees a relationship between latency and velocity such that when the latency is long, the velocity is slow. Tr. 596. He explained that the myelin controls the conduction speed, so in a demyelinating disease, where there is less myelin, the conduction is slowed. Tr. 681-82.

The nerve conduction study also measures amplitude, the strength of the electrical signal. Dorland’s at 66-67. The amplitude is proportionate to the number of nerve fibers being tested. Tr. 947. The amplitude of sensory nerves is measured in microvolts, whereas the amplitude of motor nerves is measured in millivolts. Dr. Finkel explained that it is more difficult to measure sensory nerves accurately because there is a thousandfold difference in units of measurement. Tr. 956.

Decreased amplitude indicates that the axon in the nerve is damaged. Tr. 749. If the axon is primarily damaged some of the myelin will also be lost, but this does not happen immediately. Initially, the tests will reveal decreased amplitude, and then the velocity will also begin to decrease as the myelin is subsequently damaged. Id.

There was a notable disparity in the experience of the two experts in regards to conducting and interpreting EMGs. Dr. Maertens does not usually interpret his own EMGs. Tr. 751. He relies on the interpretation of the person who conducted the test. Dr. Finkel received specialized training to perform and to interpret EMGs. Tr. 864-65. He became board-certified in the relevant discipline, electrodiagnostic

medicine, in 1999, and a primary part of his clinical practice is to conduct and to interpret pediatric EMGs. Id.; see also Exhibit O at 3. Additionally, Dr. Finkel teaches medical school residents how to perform and to interpret pediatric EMGs. Tr. 868.

**c) Electromyography #1 – February 26, 2001**

On February 26, 2001, O.A.S. had her first EMG. The doctor tested five nerves. Two of the nerves (the median sensory nerve and the median motor nerve) were in O.A.S.'s right arm. The other three nerves (two sensory and one motor) were in O.A.S.'s left leg. The sensory nerves in the left leg were the medial planar nerve and the sural nerve. Pet'r Exhibit E at 1953.

The amplitude for the nerves in O.A.S.'s arm were either normal or close to normal. The results were much different for the nerves in O.A.S.'s leg. For the medial plantar sensory nerve and the sensory sural nerve, there was no response (zero amplitude). The result for the nerve to O.A.S.'s thigh was diminished. Id.

The different nerves produced different results on velocity as well. There was no measured velocity in the two sensory nerves running down O.A.S.'s leg. The velocity in the motor nerve to O.A.S.'s thigh was strong. It measured 68 meters per second and a normal result is greater than 32 meters per second. The velocities in the nerves for O.A.S.'s upper extremities were slower than the normal result. The normal result was greater than 37 meters per second and O.A.S.'s measurements were 25 and 28 meters per second. Pet'r Exhibit E at 1953.

In conjunction with the EMG, the doctors also measured the voluntary motor unit potentials. These results showed that the most impaired part of O.A.S.'s body was her distal leg, followed by her proximal leg, followed by her hand. O.A.S.'s proximal arm (her bicep) was not affected. Pet'r Exhibit E at 1954; Tr. 963-64.

Dr. Rubin, the consultant for O.A.S.'s EMG, presented the following interpretation: "The findings are those of a length-dependent sensorimotor peripheral neuropathy, such as could be seen in inherited or metabolic neuropathies. There is no evidence of a diffuse disorder of anterior horn cells or a myopathy." Pet'r Exhibit E at 1953.

Several of Dr. Rubin's terms warrant explication.

- "Peripheral neuropathy" means that nerves outside of the brain and spinal cord are disturbed or impaired. See Dorland's at 1268 (defining "neuropathy").
- "Sensorimotor" refers to a type of neuropathy that involves both sensory nerves and motor nerves. Id. at 1269.
- "Length-dependent" means that the longer nerves are more impaired than shorter nerves. Tr. 957-58. In practical terms, a length-dependent neuropathy will appear in the lower extremities before the upper extremities.

Dr. Maertens and Dr. Finkel agreed with Dr. Rubin's finding that O.A.S.'s neuropathy was length-dependent. Tr. 582, 964.

When the Simanskis' attorney asked Dr. Maertens whether the results of O.A.S.'s February 26, 2001 EMG were consistent with a diagnosis of Guillain-Barré Syndrome, Dr. Maertens responded "[i]t really could be consistent with Guillain-Barr [sic]. It's not specific, but it could be consistent." Tr. 584.

Dr. Finkel, however, testified that the EMG was not consistent with a diagnosis of GBS or CIDP because the results did not show a significant slowing of conduction along the nerve. Tr. 964-65. Additionally, he stated that there was no evidence to support a finding of a partial conduction block or a finding of prolonged distal latency, both of which would have supported a diagnosis of GBS. Tr. 1155-57.

In addition to identifying a "length-dependent sensorimotor peripheral neuropathy," Dr. Rubin added that "[t]here is no evidence of a diffuse disorder of anterior horn cells." Pet'r Exhibit E at 1953. Dr. Finkel testified that based on what Dr. Rubin could see from the test that Dr. Rubin did not see evidence of an anterior horn cell disease. Dr. Finkel, however, explained that the testing available for anterior horn cell diseases is limited and that O.A.S.'s results alone were not sufficient to eliminate the existence of an anterior horn cell disease. Tr. 1075-78.

**d) Neuromuscular Biopsy (Sural Nerve) –  
February 28, 2001**

At the Mayo Clinic, O.A.S. had a biopsy of her sural nerve. In a biopsy, part of the body is removed for further study, usually under a microscope. Dorland's at

218. For O.A.S., the doctors removed part of the sural nerve from her left ankle. Pet'r Exhibit E at 1956. According to Dr. Finkel, the Mayo Clinic has a well-established protocol for how to obtain the nerve sample. Tr. 1082.

The nerve conduction studies from February 26, 2001, showed that O.A.S. had a length-dependent sensorimotor polyneuropathy, which means that the sensory nerves in her foot were affected. Tr. 1083. Dr. Finkel explained that "it was very reasonable to do a sural biopsy" because the Mayo Clinic was "biopsing a clinically affected nerve," which the EMG and nerve conduction tests showed was "affected along its entirety." Tr. 1084. Dr. Finkel explained that the nerve conduction studies did not reveal that "one part of the nerve was normal, [and that] the other part was abnormal." Tr. 1083-84. In other words, a sampling error was not an issue because the nerve was affected along its entirety. Id.

The result of the microscopic examination was that "[t]he myelinated fiber density is normal for age." The doctor also found "[i]ndividual epineurial perivascular inflammatory cells of unclear significance." Pet'r Exhibit E at 1956. "Perivascular" means around the blood vessels, and the "epineurium" is the covering of the fascicle, against the nerve fiber bundle.<sup>27</sup> Tr. 1080-81.

Even though this biopsy was done "blindly," meaning the doctor performing the biopsy does not know whether the area being examined had any inflammatory changes, Dr. Finkel emphasized that the Mayo Clinic did an excellent job in thoroughly teasing out the fiber presentation of the biopsy and in taking "a substantial nerve sample." Tr. 1158-59.

Dr. Maertens opined that the sural nerve is "kind of a small nerve that has very little importance." Tr. 742. He stated that the only thing to conclude from the biopsy was that "at this level on this very small segment there is no documentation or demonstration of the demyelination." Tr. 744. He posited that there could be

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<sup>27</sup> When the neurologists were asked about the inflammatory cells, neither Dr. Maertens nor Dr. Finkel suggested that they had any significance. Tr. 589 (Dr. Maertens: "it showed very little"), 1002 (Dr. Finkel: "I don't know what to make of that"). Dr. McCusker, too, stated "the clinical significance of the perivascular mononuclear cells is unclear." Tr. 516. Dr. Shoefeld, however, stated that they showed that the body's immune system was attacking O.A.S.'s nerves. Tr. 96-97, 313-14.

evidence of demyelination elsewhere, but agreed that the biopsy did not “show any evidence of demyelination.” Id.

Dr. Maertens also asserted that the sural nerve biopsy “has no value” in diagnosing GBS, CIPD, or SMARD, Tr. 780, even though the medical literature he cited in his expert report stated the opposite. The European Federation of Neurological Societies Guidelines provides that “[n]erve biopsy, usually sural sensory nerve biopsy, is considered useful for confirming the diagnosis” of CIDP. Exhibit 41, tab A (Joint Task Force of the EFNS and the PNS, European Federation of Neurological Societies/ Peripheral Nerve Society Guideline on Management of Chronic Inflammatory Demyelinating Polyradiculoneuropathy. Report of a Joint Task Force of the European Federation of Neurological Societies and the Peripheral Nerve Society, 10 J. Peripher. Nerv. Sys. 220 (2005)) at 221. Table 3 in the Guidelines lists the biopsy as “supportive criteria” for diagnosing CIDP. Id. at 223.

Dr. Finkel testified that O.A.S.’s biopsy did not support “either a focal or a more generalized demyelinating process like GBS or CIDP.” Tr. 1002. A normal result from a sural nerve biopsy is inconsistent with a diagnosis of GBS because the biopsy is “abnormal about 90 percent of the time in GBS.” Id.; accord Tr. 1159. Therefore, it is not impossible to have a normal biopsy and a diagnosis of GBS. But, a normal result is uncommon because only ten percent of patients have a normal biopsy. Tr. 1002.

#### **e) Muscle Biopsy – March 2, 2001**

A muscle biopsy was conducted on March 2, 2001. Exhibit E at 1955. A sample was taken from two muscles, O.A.S.’s gastrocnemius and her vastus lateralis. Id. The gastrocnemius is in the calf and the vastus lateralis is in the front thigh. Dorland’s at 1195.

Dr. Andrew G. Engel examined the muscle samples and diagnosed O.A.S. as having “[d]enervation atrophy, marked in gastrocnemius, slight in vastus lateralis.” Dr. Engel also commented that “[t]he etiology of the neurogenic process cannot be determined on histologic grounds.” Pet’r Exhibit E at 1955.

Dr. Maertens observed that the muscle biopsy revealed “significant denervation atrophy” in the calf muscle, and less significant denervation in the thigh muscle. He also noted that there was a lack of nerve input into the muscles.

Tr. 594. Dr. Maertens did not opine as to whether the biopsy results were more consistent with GBS, CIDP, or SMARD.

Dr. Finkel agreed with the findings and added that the biopsies supported a length-dependent process. Tr. 1090. He noted that the biopsy report did not mention a trichome stain, which would have been helpful because the stain can show whether portions of the nerves are demyelinated. Tr. 1091.

**f) Electromyography #2 (Phrenic Nerve) –  
March 6, 2001**

An EMG was performed on O.A.S.’s left phrenic nerve on March 6, 2001, at the Mayo Clinic. O.A.S.’s right phrenic nerve could not be tested because of an intravenous line (“IV”). Pet’r Exhibit E at 1952. The phrenic nerve stimulates the diaphragm, which in turn controls breathing. Tr. 680-81. Dr. Finkel stated that while it was “certainly reasonable” not to test the right phrenic nerve due to the IV, it was “a little challenging” to test only the left nerve because “it was the right diaphragm, hemidiaphragm, that was elevated.” Tr. 967.

The amplitude of the phrenic nerve was 1.0 millivolts and the distal latency was 7.1 milliseconds. Pet’r Exhibit E at 1952. How O.A.S.’s results compared to normal values was a bit complicated for the distal latency.

For the amplitude, the consultant who performed the test, B. A. Crum, reported that “[a]lthough there are no normal values for infants for our laboratory, an amplitude of 1.0 millivolts would be considered within normal limits for adults.” Pet’r Exhibit E at 1952. Dr. Finkel acknowledged that one millivolt was a normal value for adults and was willing to accept one millivolt as a normal value for infants.<sup>28</sup> Tr. 967.

For the distal latency, the consultant did not provide any normal values. See Pet’r Exhibit E at 1952. A different doctor at the Mayo Clinic, Dr. Kotagal, wrote

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<sup>28</sup> Dr. Finkel explained “We don’t have good data, however, to know what an amplitude is in an infant for the phrenic nerve. We know what it is for adults, sort of, more or less. And I would agree with the comment of Dr. Krum [sic], who did that study and prepared that report, that it’s around one millivolt . . . . I have to go with it’s probably normal, at least on the basis of adult data.” Tr. 967.

that “[m]y colleague, Dr. Kuntz, informs me that the normal latency should be <2 m[/]sec.”<sup>29</sup> Pet’r Exhibit E at 2251. Dr. Maertens accepted Dr. Kotagal’s (or Dr. Kuntz’s) information. Tr. 597-98.

Dr. Finkel came to a different opinion regarding the expected values for distal latency in infants. He stated “something around 7.1 is well within the normal range for distal latency.” Tr. 1023 (citing Exhibit QQ (Robert I. Ross Russell et al., Normal Values for Phrenic Nerve Latency in Children, 24 Muscle & Nerve 1548 (2001)) at 1549).

The Ross Russell article reported the results of its study plus the results of three other studies. For the 45 patients between zero and six months old, the mean distal latency for the phrenic nerve was “six milliseconds plus or minus 1.6.” Tr. 1022. However, as petitioners referenced in their reply brief, the Ross Russell article acknowledges that “phrenic nerve latencies are difficult to obtain in babies because of their short necks, and the nature of what is being measured.” Pet’r Reply at 11-12 (citing exhibit QQ at 1549).

The Ross Russell article referenced three other studies. The first study reported that the mean distal latency with one standard deviation was  $6.2 \text{ ms} \pm 1.2$ , the second study reported a mean of  $6.4 \text{ ms} \pm 0.8$ , and the third study reported a mean of  $2.4 \text{ ms} \pm 0.4$ . Exhibit QQ (Ross Russell) at 1549; accord Tr. 1022-23. Dr. Finkel posited that Dr. Kotagal or Dr. Kuntz was probably referencing the data from the last study when noting that the latency should be less than two milliseconds. Tr. 1022-23.

Whether O.A.S.’s distal latency was normal affected whether Dr. Maertens and Dr. Finkel viewed O.A.S. as suffering from GBS/CIDP or SMARD. Dr. Maertens accepted Dr. Kotagal’s handwritten report that O.A.S.’s latency exceeded the expected results. From the premise of an abnormal latency, Dr. Maertens reasoned that O.A.S. likely had slow conduction in her phrenic nerve. Tr. 681. Dr. Maertens explained that the slow conduction and normal amplitude were contrary to the results he would expect from a child with SMARD. Tr. 828. He also noted that phrenic nerve failure is common in GBS, but rare for CIPD. Tr. 769-70.

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<sup>29</sup> Dr. Finkel argued that this comment was “an incidental note from somebody who spoke to somebody to say that something on the order of two milliseconds was normal . . . , it didn’t say that they had lab normals.” Tr. 1041.

Dr. Finkel, on the other hand, testified that the EMG of the phrenic nerve revealed a “normal amplitude and a normal distal latency.” Tr. 966. He explained that there was no conduction value to evaluate because “you’re stimulating in the neck, and you’re measuring from the endpoint, which is the diaphragm. You’re not measuring a conduction between two points.” Tr. 967. Therefore, he concluded that the test was not supportive of any of the three diagnoses, CIPD, GBS, or SMARD. Tr. 968.

**g) Cerebrospinal Fluid Protein – March 3, 2001**

Diseases that cause inflammation in the nervous system, such as GBS and CIDP, are associated with an increased amount of protein in the cerebrospinal fluid (“CSF”). Exhibit HH at 4; see also exhibit GG at 3. Dr. Finkel and Dr. Maertens agreed that elevated protein levels in the CSF support a diagnosis of GBS or CIDP. Dr. Finkel testified that “if there is active inflammation, active demyelination going on, you expect to see elevated protein levels.” Tr. 899-900. Dr. Maertens testified that he looks to whether “the protein [is] significantly elevated.” Tr. 713. The amount of protein in the CSF can be measured by conducting a lumbar puncture. Mosby’s at 682-83, 686. During a lumbar puncture, a needle is inserted into the subarachnoid space of the spinal column in order to obtain a sample of the CSF. Id. at 683.

O.A.S.’s CSF was drawn on March 3, 2001, approximately five weeks after her respiratory distress. The result was normal (15 mg/ dl). Pet’r Exhibit E at 1943.

In Dr. Finkel’s opinion, the normal protein result makes a diagnosis of a demyelinating disease, like GBS or CIDP, less likely, although not necessarily impossible. Tr. 1164; see also Tr. 1003-04. Dr. Maertens agreed that a normal protein level was consistent with SMARD. Tr. 783. However, Dr. Maertens also asserted that O.A.S.’s cerebrospinal fluid was tested too remotely for the test results to be useful in ruling out GBS or CIDP. Tr. 600-02.

#### **4. Mercy Medical Center, Stay at Home, and Return to Mercy Medical Center – March 16, 2001 through April 24, 2001**

##### **a) Summary**

O.A.S. was transferred from Mayo Clinic back to Mercy Hospital on March 16, 2001. Upon O.A.S.'s arrival at Mercy, Dr. Napa described O.A.S.'s condition:

She was transferred on 3/16/01 and initially on arrival looked fairly improved compared to several weeks ago when she was there. She continued to have this abnormal breathing pattern suggesting a possibility of respiratory muscle weakness. This was worse when she has any exertion.

Pulmonary at rest, the patient seemed to do fairly well. She does have increased work of breathing after even normal activities like feeding and after she has any crying episode. She required small amount of oxygen, ¼ liter of oxygen, to help with the saturations especially after she gets increased work of breathing.

Pet'r Exhibit C at 311. Records dated March 21, 2001 show that a doctor at Mercy stated O.A.S. had "probable" Guillain-Barré syndrome. Pet'r Exhibit D at 1097.

O.A.S. was discharged on March 28, 2001 with the note, "Parents have learned to cope with this illness. They have been trained again in CPR. They are aware that at present we don't know for sure how her progress is going to be; however, [we] have been optimistic that her neurological status and her respiratory muscle weakness should continue to improve." Pet'r Exhibit C at 311-12.

Dr. Gavin saw O.A.S. on March 30, 2001. Dr. Gavin's report from this visit noted that O.A.S. "was doing very well on room air without O2 supplementation until just 4-5 days ago when it was noted that she would drop her saturations when she got upset and a little bit at night when she was sleeping." *Id.* at 187-89.

O.A.S. was re-admitted to Mercy on April 13, 2001 for respiratory failure. Pet'r Exhibit D at 344. She was placed on a ventilator and has remained on it since then. *See* exhibit 1 at 1456-67; exhibit 19 at 2. During this stay, she had another EMG. Pet'r Exhibit D at 628. On April 24, 2001, Dr. Napa transferred O.A.S. to

Johns Hopkins. Dr. Napa's report explained that the "lack of a definitive diagnosis has been a problem in addressing the extent of supporting the child." Id. at 334-36.

**b) O.A.S.'s Change in Respiratory Function**

O.A.S.'s medical records show that when she was transferred to Mayo on February 23, 2001, she required assistance with breathing. Pet'r Exhibit D at 1260 ("Thank you for accepting [O.A.S.], aged 3 months, weighing 5 kg, with respiratory failure on mechanical ventilation."). When she was transferred from Mayo back to Mercy on March 16, she was breathing on her own. Pet'r Exhibit C at 311-12. Her ability to breathe on her own clearly constitutes an improvement. However, she had a failure of respiration again on April 13, 2001, and was placed on a respirator. Pet'r Exhibit D at 344. She still requires this assistance years later. Exhibit 22 at ¶ 11.

Although neither Dr. Maertens nor Dr. Finkel commented upon O.A.S.'s change (improvement then decline) in respiratory function in their reports, they discussed this aspect of O.A.S.'s medical history in their testimony. To Dr. Maertens, it appeared that O.A.S. was "slowing, regressing here . . . the oxygen story here is quite alarming." Tr. 615. He stated that she had a relapse of GBS, and that relapsing GBS is recognized in the medical literature. Tr. 618-19, 630. To Dr. Finkel, O.A.S. "had a peripheral neuropathy that had a bit of a remitting and then relapsing feature to it." Tr. 1125. He maintained that she did not have GBS at any point. Id.

**c) Electromyography #3 – April 17, 2001**

On April 17, 2001, O.A.S. had a third EMG. Pet'r Exhibit D at 628. The purpose of this study was to compare O.A.S.'s current condition with the results from her February 26, 2001 EMG at the Mayo Clinic. Three motor nerves and one sensory nerve were tested. Tr. 968-69.

Dr. Jill R. Meilahn obtained no response for two of the motor nerves in O.A.S.'s left leg, but there was a motor response in the left median nerve. There was also no sensory response from O.A.S.'s left median nerve. Pet'r Exhibit D at 628-29. A note in O.A.S.'s medical records from Mercy states that "compared to EMG done [at] Mayo [on] 2/26/01 all parameters show worsening of condition." Id. at 373.

Dr. Finkel testified that the results revealed an axonal neuropathy, consistent with SMARD. He explained that the results could be consistent with a “rapidly progressive axonal loss” type of GBS, but stated that it was not consistent with a demyelinating type of GBS. Tr. 970-71.

Dr. Finkel also noted that this EMG might be “a technically limited study” because there was no sensory response in O.A.S.’s left median nerve, Tr. 969, but the EMG conducted nine days later, on April 26, 2001, did get a response. Compare Pet’r Exhibit D at 628, with Pet’r Exhibit F at 2349. He asserted that “you don’t lose it entirely and then nine days later somebody found it.” Tr. 969.

Dr. Maertens agreed that O.A.S.’s condition was worsening when compared to the results of her February 26, 2001 EMG. Tr. 621. He noted that her amplitude had dropped indicating that there is “some axonal damage” beginning to appear. Tr. 620. Dr. Maertens explained the significance of axonal damage: it is not “just demyelination anymore. The myelin has been stripped off, but now we have the axon[s] that are also degenerating.” Id.

The Simanskis maintained that O.A.S.’s worsening condition, as shown by the third EMG, combined with her previous clinical improvements were evidence that her GBS was relapsing. Tr. 1125. Dr. Finkel, however, explained that O.A.S.’s EMG results needed to be considered “within the context of her clinical situation, which was with RSV, and we know that we expect the RSV to improve, so it’s not at all surprising that the respiratory aspect improved transiently, but the underlying condition with her neuropathy did not improve.” Tr. 1126-27. Dr. Finkel concluded that O.A.S.’s three EMGs, taken together, indicated a progressive axonal neuropathy consistent with SMARD. Tr. 970.

## **5. Johns Hopkins University – April 25, 2001 through May 3, 2001**

### **a) Summary**

After O.A.S.’s third stay at Mercy, Dr. Napa transferred her to Johns Hopkins because the lack of diagnosis was a problem in caring for her. Pet’r Exhibit D at 334-36. A doctor at Johns Hopkins, Thomas Crawford, was aware that O.A.S. was previously diagnosed with infantile GBS. Pet’r Exhibit F at 2349-50. GBS was one of the disorders that the doctors at Johns Hopkins considered for O.A.S. They also wrote that O.A.S. could have spinal muscle atrophy. Id. at 2348.

On May 3, 2001, Dr. Crawford transferred O.A.S. from Johns Hopkins to the hospital at the University of Iowa. A brief summary sheet identifies the principal diagnosis as “inflammatory and toxic neuropathies.” Exhibit 4 at 28. The lengthier transfer report does not mention a particular diagnosis. Exhibit 4 at 30-32.

**b) Electromyography #4 – April 26, 2001**

According to Dr. Finkel, Dr. Crawford performs EMGs very thoroughly. Tr. 982. Unlike other EMG reports, this report begins with a clinical summary. Tr. 974.

Dr. Finkel asserted that it is “really important” to include the findings from the physical exam on the EMG report “because when you do the EMG, you want to correlate it with the findings on the physical exam.” Tr. 974. He explained that doctors should tailor the EMG to test the clinically affected areas identified in the physical exam. Tr. 982-83. Dr. Maertens, too, saw value in describing O.A.S.’s condition in the EMG report. Tr. 623.

Dr. Crawford observed that O.A.S. had “weak but antigravity power of the shoulders, elbows, wrists and finger flexor and extensors with no evident movement of intrinsic hand muscles.” Pet’r Exhibit F at 2349. Dr. Crawford’s physical examination revealed that O.A.S. had “good foot plantarflexion but no foot dorsiflexion or movement of the toes.” *Id.* Dr. Finkel stated that this observation meant O.A.S. could move her foot down but not up. Tr. 975. He concluded that O.A.S. had “pronounced distal weakness” because “she can’t bring her foot up, and she can’t move her index finger out.” *Id.* Dr. Maertens noted that the strong plantar flexion meant there was no spinal cord injury. Tr. 623.

For the sensory nerve portion of the study, Dr. Crawford tested one nerve, O.A.S.’s left median nerve, which runs along her arm into her thumb. Pet’r Exhibit F at 2349. Dr. Finkel testified that an amplitude of 8.4 microvolts was a low response, and the conduction velocity was “a little slow” at 22.7 m/s. Tr. 976.

The EMG report listed 49 m/s as the normal value for conduction velocity. Pet’r Exhibit F at 2349. Dr. Finkel, however, explained that 49 m/s was a template value for adults and that for O.A.S.’s age the conduction velocity should be “about 30 meters a second. And if there is an axonal process, it should be down maybe around 25. [O.A.S.’s was] 23. So it’s starting to – it might be truly a little slow.” Tr. 977. He maintained that O.A.S.’s conduction velocity was not significantly

slowed because in babies the conduction velocities are “slow to begin with, so it has got to be really slow to be outside of the normal range.” Id.

In addition to the one sensory nerve, Dr. Crawford tested six motor nerves. O.A.S.’s peroneal and tibial nerves were tested at both the ankle and the knee. There was no response from either nerve. Tr. 978. O.A.S.’s femoral nerve responded to the stimulation, but it had a reduced amplitude of 1.08 microvolts. Id. Dr. Finkel testified that the results revealed “a severe axonal neuropathy distally.” Id. Additionally, he stated that the reduced amplitude in the femoral nerve was consistent with a length-dependent, axonal process. Id.

O.A.S.’s ulnar nerve was stimulated in four locations (wrist, distal elbow, proximal elbow, and axilla). The multiple measuring points allowed Dr. Crawford to calculate a conduction velocity in three segments. The values were very similar (25.0, 22.7, and 22.2 m/s). Pet’r Exhibit F at 2349.

Dr. Finkel observed that “this is the first example where anybody has stimulated the nerve at three sites – actually at four sites, but you have three segments, and they’re all the same. There is no segmental slowing.” Tr. 980. He explained that “you would expect to see [segmental slowing] in GBS or CIDP” and “it’s not there” in O.A.S. Id.

Dr. Finkel also maintained that there is not a sampling issue here because O.A.S.’s muscle was clinically affected. The ulnar nerve “had to be affected because she didn’t move her first dorsal interosseous. And the ulnar nerve goes to the first dorsal interosseous. So we know that that was a clinically affected muscle, very affected. She didn’t move it. And yet there was no demyelination in that nerve.” Tr. 981-82.

The first dorsal interosseous is “the muscle in the hand that moves your index finger in and out.” Tr. 964; see Dorland’s at 1194, 1206. Dr. Finkel explained that the muscle is “an abductor, so it brings [the index finger] out to the side.” Tr. 975. Dr. Finkel opined that Dr. Crawford knew the muscle was clinically affected because Dr. Crawford observed that O.A.S. had “no evident movement of intrinsic hand muscles.” Tr. 974-75 (citing Pet’r Exhibit F at 2349).

Dr. Maertens testified that the conduction velocity was “definitely slow” and agreed that the results did not indicate segmental demyelination. Tr. 1236-37. He maintained that the results were more consistent with a “diffuse disease that you

see in CIDP, and the longer the disease goes the more diffuse the [disease] is, so to me it's not segmental, but it's neither distal, and it's very diffuse." Tr. 1236.

The amplitudes for the four points tested in O.A.S.'s ulnar nerve were 0.39, 0.09, 0.27, and 0.33. Pet'r Exhibit F at 2349. Dr. Finkel testified that a normal amplitude should be 6 and concluded that O.A.S. had "a very reduced amplitude." Tr. 980. The amplitudes for O.A.S.'s median nerve were 0.09 at the wrist and 0.08 at the axilla. Pet'r Exhibit F at 2349. Dr. Finkel explained that the amplitudes for the median nerve in someone O.A.S.'s age should be 4. He rounded O.A.S.'s results to 0.1 and observed that, even then, 0.1 versus 4.0 was "a huge reduction."<sup>30</sup> Tr. 979.

Dr. Crawford's interpretation was that O.A.S.'s "study demonstrates the presence of widespread neuropathic weakness. The pattern is consistent with either a motor neuronopathy or a sensorimotor axonal neuropathy." Pet'r Exhibit F at 2350. Dr. Crawford's description of O.A.S.'s problem as an "axonal neuropathy" places her injury within the axon.

Both Dr. Maertens and Dr. Finkel agreed with Dr. Crawford's interpretation. Tr. 645-46, 983-84. Dr. Maertens stated that decreasing amplitudes are indicative of an axonal injury. Tr. 624. He further explained that a motor neuronopathy means that the muscle is involved and "the anterior horn cell[s] that go to the muscle have been undergoing some degeneration due to the axonal damage." Tr. 625. Dr. Maertens concluded that O.A.S.'s condition was deteriorating and that at some point her nerves would burn out and she would be unable to recover. Tr. 626. Dr. Finkel testified that SMARD is considered both a motor neuronopathy and a sensorimotor axonal neuropathy. Tr. 984.

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<sup>30</sup> Dr. Finkel noted that O.A.S.'s median nerve was tested at Iowa nine days earlier at Iowa and "there was a response of 0.6 millivolts. So in that person's hands, it was .6. In Dr. Crawford's hands, it was rounding it off to .1." Tr. 979; see Pet'r Exhibit D at 628. He stated that the discordant results were more likely due to technical difficulties. Tr. 979.

In actuality, the testing of O.A.S.'s median nerve at Iowa was nine days after (not before) the testing at Johns Hopkins. Dr. Finkel likely misspoke. Nevertheless, the results were discordant, and, according to Dr. Finkel, likely due to technical difficulties.

## 6. University of Iowa – May 3, 2001 through August 19, 2001

### a) Summary

From Johns Hopkins, O.A.S. went to the University of Iowa, where she remained for more than three months. Once more, she had an EMG. Exhibit 5, Vol. 1, at 131. One of O.A.S.'s neurologists, Dr. Katherine Mathews, wrote a thorough report about O.A.S.'s history on May 8, 2001. In part, Dr. Mathews stated that O.A.S.'s "clinical picture is not compatible with spinal muscle atrophy." Exhibit 5, Vol. 1, at 278.<sup>31</sup> Dr. Mathews parenthetically noted that "DNA testing has been negative." DNA testing, though, "was never done nor was blood ever sent to Germany," and Mrs. Simanski ultimately decided not to pursue any genetic testing. Exhibit 22 ¶ 9, 10.

On June 30, 2001, Dr. Mathews recorded information about O.A.S.'s improving clinical picture. She indicated:

O.A.S. is clearly getting stronger. She kicks her legs very actively. She continues to have flail feet. She moves her upper extremities with good strength. Sensation in the feet is uncertain on exam today. She is so active that it is difficult to discern a difference when her feet are touched or scratched.

Exhibit 5, Vol. 3, at 1254. On this date, Dr. Mathews also consulted Dr. Sladky from Atlanta, Georgia. Dr. Sladky told Dr. Mathews that he "favors a diagnosis of an acute axonal neuropathy." Id.

The discharge summary from Iowa includes O.A.S.'s January 26, 2001 vaccinations in her history but does not ascribe a causal connection to them. Exhibit 5, Vol. 1, at 90-93.

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<sup>31</sup> She did not, however, specify how O.A.S.'s clinical course was inconsistent with SMA. Exhibit 5, Vol. 1, at 278.

**b) Electromyography # 5 – May 8, 2001**

O.A.S. had her fifth EMG on May 8, 2001. Exhibit 5, Vol. 1, at 131. The report stated that “these findings favor the diagnosis of peripheral neuropathy over motor neuron disease.” Id.

Both Dr. Finkel and Dr. Maertens explained that the results from this EMG were fairly consistent with O.A.S.’s results from the fourth EMG conducted on April 26, 2001. Dr. Maertens stated that the results were “very much the same” and “not terribly different.” Tr. 633. He testified that CIDP is a peripheral neuropathy, whereas SMA is a motor neuron disease that affects the anterior horn cell. Tr. 634. Dr. Finkel testified that the results did not “show anything particularly different.” Tr. 990. He agreed that the results were consistent with a peripheral neuropathy, but noted that the results were still indicative of a length-dependent process. Tr. 991-92.

**7. Return to Mercy Medical Center – August 20, 2001 through September 11, 2001**

O.A.S. returned to Mercy Medical Center on August 20, 2001 and remained there until September 11, 2001. Exhibit 2, Vol. 3, at 1304, 1315. When she was admitted, her diagnosis was “Flaccid Axonal Neuropathy.” Pet’r Exhibit D at 696. The Mercy discharge report, like the discharge report from Iowa, mentions that O.A.S.’s doctors recommended continued vaccinations, except that her parents did not want her to receive additional doses of pertussis vaccine. Exhibit 2, Vol. 3, at 1305-06.

**8. Return to Mayo Clinic in September 2003**

About 18 months later, Dr. Gavin recommended that the Simanskis return to the Mayo Clinic in September 2003. Exhibit 19 at 371. O.A.S. was admitted to that facility on September 15, 2003. Exhibit 3 at 19.

After she was admitted, O.A.S. had another EMG. Exhibit 3 at 10. Dr. Kuntz interpreted the results showing “a severe, diffuse sensorimotor peripheral neuropathy characterized primarily by axonal loss. There has been significant progression of findings since the prior examination dated February 26, 2001.” Dr. Maertens and Dr. Finkel basically agreed with these opinions. Tr. 645-46, 998-99.

The admission notes indicate that the doctors wanted more genetic testing. Dr. Kuntz suggested that O.A.S. had SMA/SMARD. Exhibit 3 at 29; see also exhibit 1, Vol. 3, at 1481 (duplicate). In another note, Dr. Kuntz stated “[Question] SMARD.” Exhibit 3 at 71; see also Tr. 1144-45 (Dr. Finkel’s testimony about this entry). Dr. Kuntz recommended that O.A.S. and her parents send genetic material to a doctor in Germany who was investigating SMARD.

Although not entirely clear from the medical records, the Simanskis did not send materials for genetic testing. Compare exhibit 22 with exhibit 5, vol. 1, at 278. In an affidavit prepared for this litigation, Ms. Simanski explained that in 2003, their “insurance would not pay for the genetic testing. The testing was never done nor was blood ever sent to Germany.” Exhibit 22 ¶ 9. Ms. Simanski’s affidavit also states she recently asked O.A.S.’s pediatric neurologist about genetic testing. Ms. Simanski averred that “[s]ince there is no benefit for [O.A.S.], we have decided not to seek genetic testing.” Id. ¶ 10.<sup>32</sup>

Even without the results of genetic testing, Dr. Kuntz diagnosed O.A.S. with SMARD. She wrote in her report:

Our nerve physiologic testing here demonstrated diffuse fibrillation potentials and decreased numbers of motor unit potentials along with virtually absent sensory potentials with decrease in the motor compound muscle action potential amplitudes. All of this suggests progressive motor and sensory neuronopathy or axonopathy. I believe that this is compatible with a recently described entity called spinal muscular atrophy with respiratory distress or SMARD. . . . I believe that it would be very critical for us to confirm the diagnosis for [O.A.S.].

Exhibit 1, Vol. 3, at 1481. Dr. Finkel explained that SMARD did not hit the clinical radar for most neurologists until 2003, at which point Dr. Kuntz changed her diagnosis. Tr. 906.

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<sup>32</sup> Through counsel, the Simanskis declared that they did not consent to genetic testing. See Pet’r Status Rep’t, filed July 25, 2012. The respondent could have filed a motion to compel genetic testing. See order, filed Aug. 10, 2012. The Secretary, however, declined. See Resp’t Status Rep’t, filed Aug. 20, 2012.

## 9. Treatment after September 2003

Even without confirming genetic tests, Dr. Kuntz's suggestion that O.A.S. suffered from SMARD was influential. O.A.S.'s pediatrician, Dr. Gavin, stated in the first paragraph of a request to an insurance company to provide additional support for O.A.S. that O.A.S. suffered from spinal muscular atrophy with respiratory distress. Dr. Gavin wrote this letter on November 11, 2003, approximately two months after Dr. Kuntz's report. Exhibit 1, Vol. 3, at 1456-57. However, on October 25, 2004, Dr. Gavin, again in the context of requesting services from an insurance company, stated: "[o]ne medical consultant has suggested she may have Spinal Muscle Atrophy with Respiratory Distress but this diagnosis has yet to be confirmed." Exhibit 1, Vol. 2, at 891.

In addition to Dr. Gavin, other doctors accepted the SMARD diagnosis. For example, after O.A.S. returned to Mercy Medical Center in February 2004, she was again cared for by Dr. Napa, the pediatric intensivist. In the summarized history portion of his discharge report, Dr. Napa stated "[k]nown neuromuscular disorder – SMA-RD type." Exhibit 1, Vol. 3, at 1192. In January 2007, Dr. Kabbani, O.A.S.'s new pediatric neurologist, also stated that O.A.S. had "a clinical diagnosis of sensorimotor axonal neuropathy that also can be called spinomuscular atrophy with respiratory distress."<sup>33</sup> Exhibit 20 at 2.

In October 2008, O.A.S. needed to see a new pediatric pulmonologist because her previous specialist had moved. In recounting O.A.S.'s lengthy history, Dr. Flores stated "[a]ssessment is that she has Spinal Muscular Atrophy with Respiratory Distress." Exhibit 19 at 107. Similarly, O.A.S.'s SMA was included as part of her history of present illness when she went to Blank Children's Hospital in August 2011. Exhibit 19 at 250. O.A.S.'s treating physicians have consistently referenced SMARD as the proper diagnosis since 2003.

Presently, O.A.S. still requires assistance with her breathing. A May 25, 2012 letter from Dr. Gavin to an insurance company stated that O.A.S. has "generalized, severe neuromuscular weakness due to spinal muscular atrophy and

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<sup>33</sup> O.A.S. started seeing Dr. Kabbani because in September 2006, her mother told Dr. Gavin that she preferred that O.A.S. not see Dr. Kuntz from the Mayo Clinic, Dr. Matthews from the University of Iowa, or Dr. Narawong from Mercy. Exhibit 19 at 362, 388, 504.

is on ventilator support via tracheostomy.” Exhibit 19 at 2. A July 16, 2012 report from a physical therapist states that O.A.S. has “muscle weakness, limited [range of motion] in [upper extremities] for daily tasks, decreased sitting balance, decreased strength and decreased fine motor and self-care skills.” O.A.S. uses a wheelchair. The therapist’s goals were to improve O.A.S. communication skills by activating a yes/no switch and to improve O.A.S.’s ability to track visually a falling balloon. Exhibit 38 at 45-46. O.A.S. attends school. See exhibit 23 at 1-25 (Individualized Education Plan, dated Sept. 7, 2012). Her mother states that O.A.S. “has a wonderful disposition . . . and has high cognitive functioning with some developmental delay.” Exhibit 22 ¶ 11.

### III. STANDARDS FOR ADJUDICATION

In determining whether petitioners are entitled to compensation, the statute directs special masters to consider “the entire record and the course of the injury, disability, illness, or condition.” 42 U.S.C. § 300aa—13(b)(1)(B). Medical professionals follow the same approach when assessing a patient. Dr. Finkel explained that it is necessary to look at all of the available information when making a diagnosis. Tr. 1080.

Here, the “entire record” includes the histories provided by the Simanskis, the results of tests ordered by O.A.S.’s doctors, and the doctor’s interpretation of those test results. The treating doctors’ views are “favored.” Capizzano v. Secretary of Health & Human Servs., 440 F.3d 1317, 1326 (Fed. Cir. 2006). However, the Vaccine Act states that any diagnosis “shall not be binding on the Special Master.” 42 U.S.C. § 300aa—13(b)(1).

The record also includes the numerous reports written by Doctors Shoenfeld, Maertens, McCusker, and Finkel as well as their testimony. When considering the weight to assign this opinion testimony, special masters may consider the reliability of their opinion. Moberly v. Secretary of Health & Human Servs., 592 F.3d 1315, 1329 (Fed. Cir. 2010).

When the nature of the vaccinee’s injury is in dispute, the special master must first identify the petitioner’s injury before conducting an Althen causation analysis. See Lombardi v. Sec’y of Health & Human Servs., 656 F.3d 1343, 1353 (Fed. Cir. 2011); Broekelschen v. Sec’y of Health & Human Servs., 618 F.3d 1339, 1346 (Fed. Cir. 2010). The parties agree that determining O.A.S.’s injury is the first step in determining whether the vaccinations harmed her. See Pet’r Posthr’g Br., filed May 6, 2013, at 19; Resp’t Posthr’g Br., filed June 18, 2013 at 6.

Petitioners' burden of proof is a preponderance of the evidence. Moberly, 592 F.3d at 1322; Hodges v. Sec'y of Health & Human Servs., 9 F.3d 958, 962-63 (Fed. Cir. 1993); see also Bunting v. Sec'y of Health & Human Servs., 931 F.2d 867, 873 (Fed. Cir. 1991) (holding that proof of medical certainty is not required; a preponderance of the evidence suffices).

#### **IV. WHETHER THE EVIDENCE PREPONDERATES IN FAVOR OF EITHER GBS/CIDP OR SMARD**

##### **A. Overview**

As described in the recitation of facts, O.A.S. presented normal and abnormal behaviors. Doctors also performed many tests on her. As discussed below, some signs and symptoms are consistent with SMARD and some are inconsistent, or not associated, with SMARD. The same is true for GBS/CIDP.

During the hearing both Dr. Finkel and Dr. Maertens were asked to analogize O.A.S.'s signs and symptoms to pebbles and then place each pebble on a scale. One side of the scale represents SMARD, and the other side represents GBS/CIDP. Tr. 810, 1183-85. Dr. Finkel ranked his pebbles by size (one to five with five being the most weighty) according to the amount of weight he thought should be attributed to each symptom. Tr. 1184-85. This process allowed the undersigned to weigh the evidence supporting and opposing a particular diagnosis.

##### **B. O.A.S.'s Signs and Symptoms**

###### **1. Intrauterine Growth Retardation**

When O.A.S. was born, she weighed less than five pounds. She was diagnosed with IUGR. Pet'r Exhibit A at 3; Pet'r Exhibit B at 100,102.

Dr. Maertens took different positions on the significance of O.A.S.'s IUGR. In his report, Dr. Maertens wrote that IUGR is associated with conditions other than SMARD. Exhibit 37 at 2 (citing exhibit 27 (William M. Gilbert and Beate Danielsen, Pregnancy Outcome Associated with Intrauterine Growth Restriction, 188 Am. J. Obstert. Gynecol. 1596 (2003))). Thus, according to Dr. Maertens, "at best, IUGR is neither supportive nor inconsistent with a diagnosis of SMARD1." Exhibit 37 at 2.

However, at trial, it became apparent that Dr. Maertens was using “consistent” unusually.<sup>34</sup> On cross-examination, Dr. Maertens accepted a more typical definition of “consistent” – “consistent is supportive of the diagnosis.” Tr. 769.

Dr. Maertens agreed that IUGR was “consistent with” SMARD. Tr. 765-66. Dr. Maertens maintained, though, that IUGR is not a prerequisite for SMARD. Tr. 765-66.

Later, in response to the undersigned’s question using the hypothetical scale, Dr. Maertens stated that O.A.S.’s IUGR did not go to either side of the scale. Tr. 810-11. Instead of placing it on one of the scales, Dr. Maertens stated that he would “toss” the data point. Tr. 811.<sup>35</sup>

In his report, Dr. Finkel relied upon a study showing that approximately three-quarters of patients with SMARD have intrauterine growth retardation. Exhibit HH at 2, citing exhibit S (Grohmann (2003)). Dr. Finkel wrote that IUGR is a “highly supportive feature” for SMARD. Exhibit HH at 2.

Dr. Finkel’s testimony was consistent with his report. He discussed the Guenther study that found that IUGR was 48% sensitive and 77% specific for SMARD1. Tr. 1018-19; exhibit T (Guenther) at 810, Table 1. Consequently, when asked to place the IUGR pebble on the scale, Dr. Finkel opined that IUGR was a medium sized pebble for SMARD, a size three. Tr. 1185-86.

In light of the literature indicating that many children who are later diagnosed with SMARD are born with a low birth weight, Dr. Finkel’s opinion is credited. See exhibit S (Grohmann (2003)); exhibit T (Guenther). His opinion is persuasive. O.A.S.’s IUGR supports a diagnosis of SMARD.

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<sup>34</sup> Dr. Maertens stated that “my understanding of consistent is, you know, hey, it’s something, it’s 100 percent.” Tr. 768. Later, Dr. Maertens stated “consistent means require and necessary perhaps. Perhaps.” Tr. 810.

<sup>35</sup> Dr. Maertens did not provide any testimony regarding any connection between IUGR and GBS/CIDP. See Pet’r Posthr’g Br. at 24 (discussing only Dr. Finkel’s testimony about IUGR and not citing to Dr. Maertens’s testimony about IUGR).

## 2. Acute Respiratory Failure as Presenting Sign

Before the January 26, 2001 vaccinations, O.A.S. was basically healthy. She had gained weight. See exhibit 48 (growth chart). Although she experienced an episode of gastrointestinal problems a few days before her vaccination, Pet'r Exhibit C at 194, this illness seems not to have caused any lasting consequence. However, on January 30, 2001, O.A.S. had problems breathing and was taken to Mercy Medical Center by ambulance. Exhibit 15.

The parties agree that this incident on January 30, 2001, was an episode of acute respiratory failure. See Pet'r Posthr'g Br. at 25; Resp't Posthr'g Br. at 14. The parties, however, strenuously disputed whether O.A.S.'s presentation at Mercy for respiratory failure is consistent with a diagnosis of SMARD. There is consensus that respiratory failure is typically not a presenting symptom for GBS/CIDP.

Although both SMARD and GBS/CIDP are discussed separately below, it is important to restate that the context of the analysis involves respiratory arrest as a presenting symptom. There is a difference between an initial symptom and a presenting symptom. A presenting symptom is what brings the patient to see a doctor. Tr. 712, 889-90. In other words, a patient could suffer some problem for a while, yet not see a doctor until the "presenting" symptom arises. See Tr. 1035.

### a) Respiratory Arrest and SMARD

Dr. Maertens opined that the acute onset of respiratory arrest was consistent with SMARD. An exchange with the Secretary's counsel who was cross-examining Dr. Maertens went as follows:

Q Isn't it true that respiratory arrest is typically the presenting symptom of SMARD?

A Yes.

\* \* \*

Q So now, based upon that new understanding [of the word consistent], is acute onset of respiratory failure highly consistent with SMARD?

A Yes.

Tr. 767.

Like Dr. Maertens, Dr. Finkel linked an acute presentation of respiratory problems with SMARD. He wrote that “SMARD often presents with acute respiratory failure that is triggered by an upper respiratory infection, such as RSV in [O.A.S.’s] case.” Exhibit N at 1. His testimony was similar. Tr. 935-36.

Despite the testimony from Dr. Maertens that SMARD often presents as acute respiratory failure, the Simanskis challenged Dr. Finkel on cross-examination. See Tr. 1049-64. As summarized in their brief, the Simanskis view the literature as indicating that “every child who was diagnosed with SMARD with a known gene mutation had the onset of symptoms prior to presentation of respiratory arrest.” Pet’r Posthr’g Br. at 25. The universality of symptoms before respiratory arrest is important to the Simanskis because, according to them, O.A.S. did not have any problems before her respiratory arrest. See id. at 25-27.

There are two flaws in the Simanskis’ position. The first problem stems from the Simanskis’ assertion of what happens in all cases of SMARD. The second problem concerns what happened to O.A.S.

The first concern is the onset of SMARD generally. It is difficult to accept the proposition that absolutely every child with SMARD has been noted to suffer some relatively benign symptoms before presenting to a doctor with respiratory arrest. After the Simanskis’ attorney had discussed several reports of children who had developed SMARD, she started to make the argument, which appears in their post-hearing brief, that every child with SMARD had some symptoms before the onset of respiratory arrest. But, Dr. Finkel informed the Simanskis’ attorney: “I don’t think we went through every case that was in every report. We went through certain cases that you selected and you wished to address, but let’s be clear here. We didn’t go through every case in every report.” Tr. 1148.

The medical literature about how SMARD appears initially seems to indicate more variability in presentation than the Simanskis argue. “Early-onset respiratory distress is the cardinal feature, presenting between 1 and 6 months of age, although a weak cry, inspiratory stridor, or foot deformities *may* have been noted earlier.” Exhibit Z (Eppie M. Yiu and Monique M. Ryan, Genetic Axonal Neuropathies and Neuronopathies of Pre-Natal and Infantile Onset, 17 J. Periphe. Nerv. Sys. 285 (2012)) at 289 (emphasis added). Thus, the sweeping argument made by the Simanskis’ attorney that all cases of SMARD always have some symptoms before respiratory arrest seems overdone.

The second flaw in the Simanskis' argument relates to what happened to O.A.S. They maintain that O.A.S. was healthy before her respiratory arrest on January 30, 2001. Some evidence supports this view, including a February 7, 2001 report to a doctor at Mercy Medical Center, stating O.A.S. "had a healthy cry." Pet'r Exhibit D at 1279. Other supporting evidence includes Dr. Maertens's interpretation of the December 28, 2000 video. Tr. 666, 1238.

But, other evidence contradicts this assessment. A nurse at Mercy Medical Center recorded that after O.A.S. extubated herself on January 31, 2001, she had a weak cry. Pet'r Exhibit D at 1668. Moreover, Dr. Finkel's interpretation of the December 28, 2000 video was that O.A.S.'s cry was weak. Tr. 933, 1149.

Evaluating the strength or weakness of O.A.S.'s cry is difficult. The videotape shows O.A.S. for just a few minutes and any conclusion drawn from such a limited sample could easily be mistaken. In addition, the Simanskis' own judgment about the strength or weakness of a baby's cry depends, at least in part, on their experience with other infants' cries.

On the whole, although Dr. Finkel assigned O.A.S.'s respiratory arrest as a strong pebble on the side of SMARD, a size four; Tr. 1185-86, the Simanskis have effectively cast some doubt on this point. O.A.S.'s health preceding her respiratory arrest and her respiratory arrest are generally consistent with SMARD. Ultimately, whether these pieces of information favor SMARD or GBS/CIDP depends upon the other side of the hypothetical scale.

#### **b) Respiratory Arrest and GBS/CIDP**

In Dr. Maertens's report, he wrote that "[a]cute respiratory failure is a frequent complication in patients with severe neuromuscular disease." Exhibit 37 at 1. He cited two studies in support of this statement.

In the first study, researchers were attempting to determine whether electrophysiological tests of the phrenic nerve in GBS patients could predict the severity of respiratory problems. In conducting their experiment, the researchers excluded any patients who were oxygenated or mechanically ventilated. Exhibit 29 (H. Ito, et al., Phrenic Nerve Conduction in the Early Stage of Guillain-Barre Syndrome Might Predict the Respiratory Failure, 116 *Acta Neurol. Scand.* 255 (2007)).

The second article presented case reports of “four patients with CIDP who presented with phrenic nerve palsy.” The authors state that “[d]yspnea occurred 3 months, 6 months, 9 months, and 6 years after the onset of CIDP symptoms in cases 2, 3, 4, and 1, respectively.” Exhibit 11, tab A (Tanya Stojkovic, Phrenic Nerve Palsy as a Feature of Chronic Inflammatory Demyelinating Polyradiculoneuropathy, 27 *Muscle & Nerve* 497 (2003)).

These articles provide at least some support for Dr. Maertens’s statement that patients with severe neuromuscular diseases can develop respiratory problems as a “complication” to their disease. The Stokjkovic article indicates that CIDP’s progression can include breathing disorders after at least three months. But, O.A.S.’s presentation is much different. Her first significant problem was respiratory arrest.

Dr. Maertens opined that O.A.S.’s January 30, 2001 respiratory arrest did not mark the beginning of her neurological problem (regardless of whether that disease was GBS or CIDP). He stated the onset of her demyelinating polyneuropathy “may not have been the very first day [when] she went into respiratory failure.” Tr. 669. Later, he testified that O.A.S. did not start to recover from her RSV infection after being hospitalized for a week, “that’s when things started to appear. And so I think that the Guillain-Barré syndrome probably came on during that period of time.” Tr. 673; accord Tr. 738.

Dr. Finkel, too, stated that acute respiratory failure is usually not the presenting problem in either GBS or CIDP. Tr. 902, 936; see also exhibit II (Eman F. Halawa, Guillain-Barré Syndrome as a Prominent Cause of Childhood Acute Flaccid Paralysis in Post Polio Eradication Era in Egypt, 15 *Eur. J. Paediatric Neurol.* 241 (2011) at 243, Table 2; exhibit MM (Monique M. Ryan, Childhood Chronic Inflammatory Demyelinating Polyneuropathy: Clinical Course and Long-Term Outcome, 10 *Neuromuscular Disord.* 398 (2000)) at 399.

Given that Dr. Maertens did not argue that O.A.S.’s presentation for acute respiratory failure was part of her demyelinating neuropathy, this particular piece of information does not support the diagnosis of GBS/CIDP.

### **c) Finding Regarding Acute Respiratory Failure as Presenting Sign**

As mentioned earlier, Dr. Finkel believed that O.A.S.'s presentation with acute respiratory failure strongly favored SMARD. Dr. Maertens stated that it was a "toss up finding" that should not be placed on either side of the scale. Tr. 811.

The more persuasive evidence suggests that the presentation with respiratory failure is more common in SMARD, than in GBS/CIDP. Thus, this fact about O.A.S. slightly favors the SMARD diagnosis.

### **3. Onset of Respiratory Failure at 12 Weeks**

The acute presentation of respiratory arrest relates to the question of O.A.S.'s age when she had respiratory arrest. She was born in late-2000, and suffered the respiratory arrest on January 30, 2001. Between those dates, 89 days (or 12-and-one-half weeks) elapsed.

Dr. Finkel opined that O.A.S.'s respiratory failure at her age was consistent with SMARD, but inconsistent with GBS and CIDP. Tr. 936-37, 1185, see exhibit R (Katja Grohmann et al., Mutations in the Gene Encoding Immunoglobulin  $\mu$ -binding Protein 2 Cause Spinal Muscular Atrophy with Respiratory Distress Type 1, 29 Nat. Genet. 75 (2001)) at 76 (Table 1, listing age of onset from 4-18 weeks); exhibit 28 (Tyler Mark Pierson et al., Infantile-Onset Spinal Muscular Atrophy with Respiratory Disress-1 Diagnosed in a 20-Year-Old Man, 21 Neuromuscular Disord. 353 (2011)). Dr. Finkel asserted that O.A.S.'s manifestation at six weeks to six months would be a size five pebble for SMARD. Tr. 1186. He stated that "GBS can occur at all ages but is decidedly uncommon in infants."<sup>36</sup> Exhibit HH at 3.

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<sup>36</sup> Dr. Finkel testified that he has been "referred a lot of patients with suspected GBS or CIDP." Tr. 890. He asserted that he cannot recall seeing any patient less than 18 months with GBS. Id. Dr. Finkel stated that he may have seen it, "but it would be rare." Id. Dr. Maertens testified that the majority of his GBS patients are walking, and that in the last ten years he has only followed one patient with GBS less than six months of age. Tr. 710. Dr. Maertens also opined that many people believe that GBS does not exist in infants. Tr. 716.

Dr. Maertens's opinion regarding O.A.S.'s age of onset varied. In his report, he disagreed with Dr. Finkel's characterization that O.A.S.'s age at onset was inconsistent with GBS and highly inconsistent with CIDP. Exhibit 26 at 2; see also Tr. 776-77. He stated "GBS occurs at all ages." Exhibit 37 at 2. In support of this statement, Dr. Maertens cited a 1977 case report of a 1-month-old girl whom the authors described as having GBS. Exhibit 31 (Richard C. Gilmartin & Lawrence T. Ch'ien, Guillain-Barré Syndrome with Hydrocephalus in Early Infancy, 34 Arch. Neurol. 567 (1977)).<sup>37</sup> In any event, this article actually supports Dr. Finkel's opinion because Dr. Gilmartin stated that GBS "has, not to our knowledge, been reported in early infancy." Id.

Then, at the hearing, Dr. Maertens testified that it is "extremely rare" to have the age of onset be two months in GBS. Tr. 777. He also stated that the onset of respiratory failure at two months could occur with SMARD. Tr. 813.

In short, the Simanskis have presented some evidence that an onset of GBS/CIDP in a child of O.A.S.'s age could occur but any case would be "extremely rare." The Secretary, on the other hand, has established that the age of onset for O.A.S.'s problem fits what is expected with SMARD. Thus, on the whole, O.A.S.'s age of onset tends to favor SMARD, not GBS/CIDP.

#### **4. Weakness Pattern and Reflexes**

When O.A.S. arrived at the hospital, she was in respiratory arrest, probably due to an RSV infection. Her diaphragm was not paralyzed. See footnote 23. Weakness in her diaphragm was evident on February 8, 2001, when a doctor ordered a fluoroscopy. Pet'r Exhibit D at 1494.

On the same day, Dr. Narawong, a neurologist at Mercy, evaluated O.A.S. He found that O.A.S. "moves all extremities well. The muscle tone is normal. Deep tendon reflexes are 2+ throughout." Pet'r Exhibit D at 1277-78. By the date of O.A.S.'s first EMG, February 26, 2001, O.A.S. developed weakness in her distal extremities. She did not have as much weakness in her proximal extremities. See Pet'r Exhibit E at 1953.

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<sup>37</sup> When asked about this article from 1977, Dr. Finkel stated that the girl's presentation of GBS was "atypical in many ways." Tr. 1202.

When O.A.S. was discharged from Mercy on March 28, 2001, she “continue[d] to have normal reflexes.” Pet’r Exhibit C at 312; Pet’r Exhibit D at 1079 (duplicate), see also Tr. 1119, 1127. However, when O.A.S. was at Johns Hopkins, Dr. Crawford tested her tendon reflexes. He found that they were absent. Pet’r Exhibit F at 2349. When O.A.S. was at the University of Iowa Hospital, there are reports that her reflexes improved. Exhibit 5, Vol. 1 at 443; see also Tr. 635-36, 1132-33, 1195.

The clinical diagnostic criteria for GBS are weakness in one or more limbs and lack of reflexes. Tr. 895-96, 1215-16. In cases of GBS, according to Dr. Finkel, “reduced or absent reflexes in particular [are apparent] at the get-go in the emergency room.” Tr. 895. Dr. Maertens stated that “in most cases [of GBS], the reflex[es] are decreased or lost.” Tr. 1229.

Consequently, the preservation of O.A.S.’s reflexes, as noted in Dr. Narawong’s report, makes the diagnosis of GBS/CIDP less likely in Dr. Finkel’s view. Tr. 1208-09. When Dr. Maertens was asked to address O.A.S.’s reflexes during her first hospitalization at Mercy, his response was an indication of 2+ reflexes, which is a normal result, “makes no sense.” Dr. Maertens “just [could not] believe that [Dr. Narawong was] right” about O.A.S.’s reflexes. Tr. 827. In reference to Dr. Napa’s March 28, 2001 discharge report about O.A.S. maintaining her reflexes, Dr. Maertens said that this data point does not favor either SMARD or GBS/CIDP. Tr. 835.

In SMARD, reflexes vary. Dr. Finkel stated “I would say we do know that the evolution of the loss of reflexes can be variable in SMARD over the course of the first few months or even years after the initial presentation with respiratory distress.” Tr. 1176. Relying upon the Giannini article, Dr. Finkel stated that “absent reflexes [are] not a necessary feature for SMARD.” Tr. 1013, 1015 (citing exhibit Q (Alberto Giannini, et al., Respiratory Failure in Infants Due to Spinal Muscular Atrophy with Respiratory Distress Type 1, 32 Intensive Care Med. 1851 (2006))). Dr. Maertens was not asked to comment on reflexes in SMARD.

In addition to the reflexes, other aspects about where O.A.S. experienced problems are helpful in distinguishing SMARD from GBS/CIDP. O.A.S. had weakness in her diaphragm before the doctors noted weakness in her distal extremities. Diaphragmatic eventration is consistent with SMARD. Tr. 1189. But, as discussed in section II.A.2., GBS/CIDP typically does not present with respiratory problems.

## 5. Progression to Permanent Ventilator Support

After O.A.S.'s respiratory arrest on January 30, 2001, she required assistance with breathing and was intubated. She extubated herself before she was transferred to the Mayo Clinic on February 23, 2001. She regained the ability to breathe on her own. See Pet'r Exhibit E at 2161 (March 14, 2001), Pet'r Exhibit C at 311, 187-89. Unfortunately, on April 13, 2001, she had another episode of respiratory arrest. Pet'r Exhibit D at 344. She still relies on a ventilator.

Whether O.A.S.'s permanent need for assistance with her breathing fits with either SMARD or GBS/CIDP are separate questions. Each is taken up below.

### a) Permanent Ventilator Support and SMARD

Dr. Finkel stated that it is typical for patients with SMARD to progress to permanent ventilator support within one month of respiratory failure. Exhibit GG at 2; Tr. 935-38. There is little doubt that patients with SMARD require ventilators to help with their breathing. In 2011, researchers reported the results of a long-term study of 11 children with SMARD who survived their first year of life.<sup>38</sup> The authors state that by nine months, all children "were mechanically ventilated." Of this group, only one was weaned, and even that girl uses a ventilator for 12 hours a day. Exhibit P (Eckhart) at e151.

The Simanskis do not challenge Dr. Finkel's opinion that SMARD patients require ventilator support. Indeed, Dr. Maertens agreed that O.A.S.'s progression to permanent ventilator support "would probably go more towards SMARD." Tr. 813.

Rather, the Simanskis argue that O.A.S. did not require ventilator support until more than 30 days after her initial episode of respiratory arrest on January 30, 2001. In their view, the approximate one-month period during which she could breathe on her own makes O.A.S.'s case inconsistent with SMARD and consistent with a relapsing case of GBS/CIDP. Pet'r Posthr'g Br. at 27-28.<sup>39</sup> On cross-

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<sup>38</sup> As noted in the article, some children with SMARD do not survive past one year.

<sup>39</sup> In the Simanskis' first brief, they stated that "[O.A.S.] did not progress to ventilator support." Pet'r Posthr'g Br. at 27. This unqualified statement is erroneous. The Simanskis' reply brief more accurately states that "[O.A.S.] did

(...continued)

examination, Dr. Finkel acknowledged the reasonableness of the Simanskis' point. See Tr. 1150-52.

**b) Permanent Ventilator Support and GBS/CIDP**

Dr. Finkel stated that permanent ventilator support was inconsistent with GBS and highly inconsistent with CIDP. Exhibit GG at 2. He expected “that there would be complete or near complete recovery in a child with GBS.” Tr. 1120.

Dr. Maertens's experience as a treating doctor was in accord with Dr. Finkel's expectations. Among his pediatric patients with GBS, he stated that perhaps one remained permanently ventilated. Tr. 755. Nevertheless, Dr. Maertens maintained that progression to permanent ventilator support “occurs with GBS and persists in CIDP if the patient is unresponsive to IVIG and/or steroids.” Exhibit 37 at 2. Dr. Finkel, too, recognized that GBS can lead to long-lasting respiratory problems and cited two articles in support. These articles indicate that approximately 20 percent of people with GBS have respiratory problems. Exhibit II (Halawa et al.) at 242 (stating “15% of GBS have persistent disability and a mortality rate that ranges from 2% to 12%. Respiratory failure is the most life threatening complication and mechanical ventilation has been reported to be needed in about 20-30% of patients.”); exhibit OO (Francis J. DiMario Jr. & Carrie Edwards, Autonomic Dysfunction in Childhood Guillain-Barré Syndrome, 27 J. Child Neurol. 581 (2012)) at 585 (respiratory failure in 15-24% of pediatric cases of GBS).

The probabilities make O.A.S.'s unfortunate need for ventilator support more consistent with SMARD, not GBS. The need for ventilator assistance, however, is not unheard of in GBS cases. A fraction of GBS-afflicted people, perhaps one in five, requires ventilator assistance. But, all patients with SMARD are aided in their breathing. O.A.S.'s progression to permanent ventilator support weighs in favor of SMARD.

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not progress to permanent ventilation within one month of respiratory failure, as the clinical picture for SMARD would suggest.” Reply at 7.

## 6. Sural Nerve Biopsy

At the Mayo Clinic, O.A.S. had a biopsy of her sural nerve. It showed normal myelination. Pet'r Exhibit E at 1956; see also Tr. 744 (Dr. Maertens: the sural nerve biopsy “doesn't show any evidence of demyelination, period”).

There is no question that the majority of biopsies from patients suffering either GBS or CIDP return abnormal results. Exhibit 41, tab A (European Task Force Guidelines) at 223 (Table 3 listing a nerve biopsy as “supportive criteria” for diagnosing CIDP). Dr. Finkel estimated that in cases of GBS, the biopsy is abnormal more than 90 per cent of the time. Tr. 1158-59. Dr. Maertens agreed. Tr. 855. Dr. Maertens recognized that if the sural biopsy “was positive it would have been helpful” in supporting a diagnosis of a peripheral neuropathy such as CIDP. Tr. 814. However, because the biopsy showed only “mild inflammation” and not any demyelination, the results did not go “in any specific direction.” Id.<sup>40</sup>

Dr. Maertens's opinion overlooks how frequently biopsies are abnormal in GBS/CIDP. It is also inconsistent with the literature that he provided. See exhibit 41, tab A at 221; Tr. 781. Dr. Finkel's conclusion that O.A.S.'s normal biopsy was inconsistent with, but not impossible for, GBS/CIDP, Tr. 1001-02, is more persuasive. While a normal biopsy is not dispositive of a diagnosis, O.A.S.'s normal biopsy would place her in the minority for GBS patients (ten percent). Dr. Finkel's opinion is credited, and O.A.S.'s normal biopsy supports a diagnosis of SMARD.<sup>41</sup>

## 7. Cerebrospinal Fluid Protein

O.A.S.'s CSF was tested on March 3, 2001. The result was normal. Pet'r Exhibit E at 1943.

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<sup>40</sup> The Simanskis argue that the sural nerve biopsy may have been normal because of a sampling error. Pet'r Posthr'g Br. at 30. Dr. Finkel effectively refuted this argument by explaining that because O.A.S. had weakness in her lower extremities, her sural nerve must have been affected. Tr. 1080-84

<sup>41</sup> O.A.S. also had a neuromuscular biopsy on March 2, 2001. Pet'r Exhibit E at 1955. The results of this test do not point to either SMARD or GBS/CIDP. See Tr. 592, 1090-91.

In only approximately ten percent of GBS/CIDP cases is the protein level normal. Tr. 1163 (Dr. Finkel discussing GBS); exhibit 16, tab 44 (Thomas Eckert et al., A Case of Influenza Vaccination Induced Guillain Barré Syndrome with Normal Cerebrospinal Fluid Protein and Improvement on Treatment with Corticosteroids, 37 Scand. J. of Infect. Disord. 621 (2005)) at 623; exhibit 41, tab A (European Guidelines) at 223. Dr. Finkel agreed that a patient does not have to have elevated protein levels for a diagnosis of GBS. Tr. 1164. He maintained, though, that a normal protein level was “certainly not consistent with general concepts about GBS” and that it would be “unusual.” Tr. 1164-65. Even though Dr. Maertens asserted that a “normal [protein level] doesn’t rule out GBS,” Tr. 739, Dr. Maertens also testified that he is quick to conduct a spinal tap in his patients because “spinal fluid is all you need to make a diagnosis of GBS.” Tr. 715.

The complicating aspect, in this case, is the timing of the CSF test. Dr. Maertens stated that the level of protein in the CSF depends “on how active the demyelination is at the time of the lumbar puncture . . . if you do it too early, you might miss it, if you do it too late, you might miss it.” Tr. 601. Dr. Kotagal’s handwritten note stated that “CSF protein elevation is maximal in the first 2 weeks of illness.” Pet’r Exhibit E at 2151.

Dr. Finkel, however, disagreed with the assertion that O.A.S.’s CSF fluid was drawn so remotely that the result did not have any diagnostic significance. He explained that the protein level “can be normal in the first few days, and it does tend to go up in the first week to ten days. . . it will build up, but it doesn’t just come back down to normal. So it’s going to be elevated for several weeks.” Tr. 1098. In Dr. Finkel’s view, if O.A.S. suffered from a demyelinating disease (either GBS or CIDP) beginning “a few weeks” before the lumbar puncture as Dr. Maertens proffered, Tr. 735-37, then she would still be undergoing demyelination on March 3, 2001. Tr. 1100.

Dr. Finkel’s testimony is consistent with the observations of Dr. Mathews, a neurologist who saw O.A.S. in May 2001. Dr. Mathews’s history states that O.A.S. “was treated for possible Guillain-Barré syndrome, however, her CSF protein was normal.” Exhibit 5, Vol. 1, at 277.

O.A.S.’s normal CSF protein level favors a diagnosis of SMARD. Dr. Finkel emphasized that only 10 percent of GBS patients have a normal protein result and concluded that a normal protein result, like O.A.S.’s, was “unlikely” for

GBS. Tr. 1164.<sup>42</sup> According to Dr. Maertens, although most cases of GBS have an elevated protein level, a GBS diagnosis does not require an elevated protein level. Tr. 601.

Although a normal CSF protein might occur in a patient with GBS/CIDP, a normal CSF protein is typical for a SMARD patient. Dr. Finkel stated that O.A.S.'s normal protein level was highly consistent with SMARD. Exhibit GG at 3. Dr. Maertens, too, testified that a normal CSF protein level is consistent with SMARD. Tr. 783. Therefore, O.A.S.'s normal CSF protein level tends to favor of a diagnosis of SMARD.

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<sup>42</sup> The Simanskis cite other articles in which the percentage of GBS patients with normal protein scores is higher than ten percent. Pet'r Posthr'g Br. at 31.

## 8. Creatinine Levels

O.A.S.'s creatinine level was tested repeatedly.

Date	Facility	Result	Normal	Comment	Citation
1/30/01	Mercy	0.1	0.3-0.5	Low	Pet'r Exhibit D at 1436
2/24/01	Mayo	0.3	Not given		Pet'r Exhibit E at 1942
3/10/01	Mayo	0.5	Not given		Pet'r Exhibit E at 1942
3/16/01	Mercy	0.2	0.3-0.5	Low	Pet'r Exhibit D at 1102
4/15/01	Mercy	0.2	0.3-0.5	Low	Pet'r Exhibit D at 394
4/29/01	Johns Hopkins	<0.1	0.5-1.2	Low	Exhibit 39 at 7
2/28/02	Mercy	0.0	0.3-0.5	Low	Exhibit 2, Vol. 4 at 1844
4/6/02	Mercy	0.0	0.3-0.5	Low	Exhibit 2, Vol. 4 at 1977
9/18/03	Mayo	0.3	0.3-0.6	Normal	Exhibit 3 at 8
2/10/04	Mercy	0.1	Not given		Exhibit 1, Vol. 3 at 1193
5/26/04	Mercy	0.2	0.3-0.5	Low	Exhibit 2, Vol. 6 at 2709
8/19/04	Iowa	0.5	0.3-1.0	Normal	Exhibit 1, Vol. 3 at 1080
5/23/07	Mercy	<0.2	0.5-0.8	Low	Exhibit 19 at 625

Dr. Finkel observed that there is “something peculiar about the Mayo Clinic lab because the two times that she was there the values, the creatinine values were clearly higher than at other facilities done just before or just after.” Tr. 1168. Dr. Finkel made this observation based upon O.A.S.'s pattern of creatinine results. Dr. Finkel testified that “if you look at the entirety of her serum creatinines from different hospitals with different labs and different normal values, I think you can see that the values are very low for the remainder of 2001 when looked at elsewhere and in 2002 elsewhere. So what I don't have is an answer why Mayo Clinic's labs seem to be higher than other hospitals.” Tr. 1167. In reference to the March 10, 2001 and March 16, 2001 results, Dr. Finkel testified that “within six days there wouldn't be a change in creatinine unless the child was dehydrated or [there was] some other reason causing the creatinine to be higher than baseline.” Tr. 1168-69.

Even if the Mayo Clinic creatinine values were accepted, Dr. Finkel still held the opinion that the results of O.A.S.'s creatinine testing are consistent with a diagnosis of SMARD. Dr. Finkel, without contradiction, explained a rise in creatinine could still be consistent with a diagnosis of SMARD because "children with SMARD can actually improve . . . they can increase in their muscle strength." Tr. 1095.

Dr. Maertens testified that an abnormally low creatinine level is consistent with a diagnosis of SMARD. He argued, however, that in O.A.S.'s case her creatinine level did not support a diagnosis of SMARD or GBS/CIDP because her creatinine levels differed throughout her illness. Tr. 783. Dr. Maertens maintained that he would "toss" the creatinine data point. Tr. 814.

Dr. Finkel, on the other hand, opined that low levels of creatinine are consistent with a diagnosis of SMARD because low creatinine indicates "decreased muscle bulk." Tr. 1091-92. Neither Dr. Finkel nor Dr. Maertens associated low levels of creatinine with GBS/CIDP.

Consequently, O.A.S.'s low creatinine level provides modest support for SMARD.

## **9. EMGs and Nerve Conduction Studies**

As summarized throughout section II.B. above, O.A.S. received many electromyograms and nerve conduction studies. That section also presented the opinions of Dr. Maertens and Dr. Finkel. Rather than repeat these summaries, this portion of the decision highlights the results of two tests – the first one, which was conducted on February 26, 2001, at the Mayo Clinic, and the fourth one, which was conducted on April 24, 2001, at Johns Hopkins.

The nerve conduction study can measure the latency, velocity, and amplitude of an electric signal. A low result for amplitude means that the axon, the part of the nerve that carries the electrical signal, is damaged. When the velocity is slow, the myelin is damaged. Tr. 681-82, 748-53, 1218.

O.A.S.'s first EMG and nerve conduction study is an important piece of information because Dr. Maertens opined that O.A.S. suffered a demyelinating process and then, after her demyelination progress, her axons were damaged. Tr. 706, see also Tr. 583, 619-20, cf. 887 (Dr. Finkel's interpretation of Dr. Maertens's opinion). In contrast, Dr. Finkel opined that O.A.S. "started with an axonal

neuropathy, and it progressed and continued as an axonal neuropathy.” Tr. 887. Thus, the difference between Dr. Maertens and Dr. Finkel begins at the first EMG and nerve conduction study.

On direct examination, Dr. Maertens interpreted the results of O.A.S.’s February 26, 2001 EMG and nerve conduction study as “a demyelinating process.” Tr. 583, 620. However, he did not elaborate on what specific portions showed demyelination.

On cross-examination, the Secretary’s counsel pressed Dr. Maertens to show evidence of demyelination on this EMG. He stated:

Well, if I look at the amplitudes, the amplitudes I believe are pretty good whenever they could be obtained, and the velocities are pretty long.<sup>[43]</sup> . . . [W]hen you lose your myelin, . . . your conduction of electricity in the nerve, become more linear and it doesn’t make little jumps and it slows down, but it doesn’t affect the nerve everywhere. It could have segment of nerve that are preserved.

Tr. 747. However, as revealed upon further cross-examination, this summary is not entirely accurate. For example, of the five nerves that were tested, the amplitude was low in four them, including two nerves that had zero amplitude. Pet’r Exhibit E at 1953. Thus, Dr. Maertens erred when he asserted the amplitudes were “pretty good.”

Dr. Finkel disagreed with Dr. Maertens’s interpretation that O.A.S.’s first EMG showed demyelination. He stated that this test showed “a very pronounced axonal, primary axonal, process. . . . [I]t’s not a primary demyelinating process with secondary axonal change. This is an axonal neuropathy with this borderline or mild slowing as a consequence of axonal loss.” Tr. 965. In his view, this first

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<sup>43</sup> Although Dr. Maertens used the word “velocities,” he later clarified that he intended to refer to the distal latencies. Tr. 750. O.A.S.’s distal latencies were prolonged as Dr. Maertens stated.

test is compatible with SMARD, but not compatible with GBS/CIDP. Tr. 964-65.<sup>44</sup>

On cross-examination, the Simanskis' attorney tried to call into question Dr. Finkel's interpretation that the EMG showed primarily axonal damage:

Q So this is kind of equivocal really as to whether she has axonal neuropathy, isn't it?

A Nope, not at all.

Tr. 1075.

Upon further questioning, Dr. Finkel also discussed the (lack of) demyelination in the February 26, 2001 nerve conduction study. He stated "it was not significantly slowed. It was consistent with the type of mild slowing you would see from an axonal process, but not demyelinating." Tr. 1088. The Simanskis' attorney persisted:

Q And based on this exam, you can't really say there is no segmental demyelination, though, can you?

A Well, in the nerves that are there, there is no evidence to support that. Maybe there are other nerves that had it, but I'm just going on what we have.

Q Okay. And that's what I was asking.

A I'm not going to exclude – I mean, as they say, anything is possible. But if I'm being asked to address based on the data in front of me, no, there is no evidence for segmental demyelination.

Tr. 1090.

After Dr. Finkel testified, Dr. Maertens was asked to address this EMG again. He testified:

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<sup>44</sup> The interpreting doctor at the Mayo Clinic, Dr. Rubin, stated that there was "no evidence of a diffuse disorder of anterior horn cells." Pet'r Exhibit E at 1953. The views of the treating doctors are analyzed below.

THE COURT: So do you think that this EMG supports the GBS diagnosis?

THE WITNESS: Well, the EMG is done here. It doesn't disqualify GBS, but it doesn't go either way. It suggests that there is already some axonal problem at this time of the study, but I don't think it really says that – it's not a complete study, so we don't really have an idea if there is segmental demyelination or not, and we really cannot tell either way.

THE COURT: Did you just say that it shows an axonal process?

THE WITNESS: I think so, yes.

THE COURT: Okay. And what part of it shows an axonal process?

THE WITNESS: The amplitude is somewhat decreased. It's very mild, but in some situations, it seems like in the peroneal [nerve] we have a more significant reduction, so there is some indication of axonal damage.

THE COURT: Is there any indication of demyelinating damage on this EMG?

THE WITNESS: Well, the velocities are decreased, and it's not totally inconsistent with demyelination nor inconsistent with axonal damage. So it goes both ways.

Tr. 1233-34.<sup>45</sup>

In sum, both Dr. Maertens and Dr. Finkel interpreted the first EMG as showing some axonal damage. But, there was some disagreement as to whether this EMG showed demyelination. Dr. Finkel stated that he did not see any evidence of demyelination and Dr. Maertens said the results were “not totally inconsistent with demyelination.” Thus, additional evidence regarding demyelination must be sought from other EMGs.

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<sup>45</sup> The Simanskis do not address this portion of Dr. Maertens's testimony. Instead, they cite Dr. Maertens's testimony at transcript page 620. See, e.g., Pet'r Posthr'g Reply at 10.

As the doctors continued to attempt to determine what was causing O.A.S.'s problems, they conducted additional EMGs and nerve conduction studies. Dr. Crawford from Johns Hopkins conducted a test on April 26, 2001. Pet'r Exhibit F at 2349. Both Dr. Maertens and Dr. Finkel complimented this test. Tr. 623, 974, 976, 983.

When he looked at the results, Dr. Maertens saw “[d]emyelination, but now we have also amplitude that is really going downhill and that’s more an axonal injury now.” Tr. 624. In his view, O.A.S.'s health was declining as her nerves burned out. Tr. 626.

Dr. Finkel noticed the reduced amplitudes and concluded that the process affecting O.A.S.'s nerves was axonal. Tr. 978, 980, 983. Dr. Finkel stressed that this nerve conduction study was significant because it was “the first example where anybody has stimulated the nerve at three sites – actually at four sites, but you have three segments.” Tr. 980. Essentially, Dr. Crawford divided O.A.S.'s ulnar nerve into three segments, by making marks at O.A.S.'s wrist, distal elbow, proximal elbow, and axilla. Dr. Crawford was able to determine the conduction velocity for each of these three segments and the results were 25.0, 22.7, and 22.2 meters per second. Pet'r Exhibit F at 2349. In Dr. Finkel's words, these measurements are “all the same. There is no segmental slowing.” Tr. 980.

Segmental slowing is present in cases of GBS and CIDP. Tr. 980-81, 1084-86. In contrast, for SMARD, the slowing is “uniform. . . . It's not segmental slowing.” Tr. 987. Consequently, Dr. Finkel found this nerve conduction study as supporting SMARD, and not supporting GBS/CIDP. Tr. 983-84.

In rebuttal, Dr. Maertens was again asked to address Dr. Finkel's interpretation that there was no segmental slowing. Dr. Maertens testified: “so to me it's not segmental, but it's neither distal, and it's very diffuse. It's definitely not a distal, axonal damage.” Tr. 1236.

Given that there is segmental slowing in GBS/CIDP and given that Dr. Maertens conceded that this EMG did not show segmental slowing in O.A.S., the logical deduction is that O.A.S. did not have GBS/CIDP.

## **10. Response to IVIG Treatment**

O.A.S. had her first IVIG treatment at Mayo in March 2001. Tr. 1101; Pet'r Exhibit E at 2158. Dr. Finkel and Dr. Maertens agreed that it was appropriate to

give O.A.S. IVIG treatments even though her CSF protein was not elevated. Tr. 898. Dr. Finkel stated “if we have a strong clinical suspicion and we’ve excluded other things along the way, then we start the IVIG.” Tr. 898. Dr. Maertens explained that “[y]ou have a degree of suspicion, you go on the base [sic] of your suspicion, you treat. If you see improvement, you are good.” Tr. 737. Dr. Maertens also testified that most children with GBS respond well to IVIG treatment. Tr. 720.

After this treatment, O.A.S.’s “[m]other believe[d] in past 2 days that [O.A.S.] has ‘turned the corner’ – She is more comfortable when exerted, moves more spontaneously.” Pet’r Exhibit E at 2158. She was successfully extubated. Dr. Kuntz wrote: “Improved head control. Will expect continued gradual improvement in strength. Will sign off.” Pet’r Exhibit E at 2160. O.A.S. was transferred back to Mercy, at which point she was discharged home. Tr. 1101-02.

After O.A.S. was re-admitted to Mercy with respiratory failure in April 2001, she received her second IVIG treatment. Tr. 1171; Pet’r Exhibit D at 377. On April 19, 2001, she could shrug her shoulders, but she did not have movement in her lower extremities. Pet’r Exhibit D at 370.

While at the University of Iowa in May 2001, O.A.S. received a third IVIG treatment. Exhibit 5, Vol. 1, at 465. Dr. Mathews noted in the assessment section of her report that O.A.S.’s “reflexes are definitely coming back.” Exhibit 5, Vol. 1, at 443, 466. However, in the physical examination section of the same report, Dr. Mathews wrote “[O.A.S.] is essentially unchanged.” Exhibit 5, Vol. 1, at 443. Dr. Finkel argued that “the changes here are by parental report and that Dr. Mathews’ [sic] exam actually saw little change.” Tr. 1133.

On June 30, 2001, Dr. Mathews notes that “[O.A.S.] is clearly getting stronger. She kicks her legs very actively. She continues to have flail feet.” Exhibit 5, Vol. 3, at 1254. Dr. Finkel explained that Dr. Mathews’s note indicated “there was improvement in the proximal strength in the lower limbs, not distally where there was still flail, and that her arms seem to be moving more actively as well.” Tr. 1135-36. Dr. Finkel testified that after the IVIG treatments, O.A.S. showed “some marginal signs that hinted at some improvement.” Tr. 1130; accord 1171.

Dr. Maertens testified that O.A.S.’s improvement after the IVIG treatments strongly supported a diagnosis of GBS. Tr. 858. He explained that O.A.S. “responded to the first treatment, and she responded again a little bit to the second

treatment, but definitely to the first treatment she responded to the point of coming off the vent. She really did improve remarkably. And you would not expect any response to treatment with SMARD, with IVIG.” Id. The Simanskis put forward this argument. Pet’r Posthr’g Br. at 23, 33. Dr. Maertens concluded that most cases of GBS resolve and that most children respond positively to IVIG treatment. Tr. 717, 720.

Dr. Finkel agreed that O.A.S.’s condition improved following the first treatment with IVIG at the Mayo Clinic, but maintained that the improvement “was largely due to the improvement in the RSV, not the underlying peripheral neuropathy.” Tr. 1171, accord Tr. 1126-27.

This data point presents a closer call. After O.A.S. received the first course of IVIG, she did not need assistance from a ventilator. However, it is less clear that she improved because of the IVIG. O.A.S.’s concurrent recovery from the RSV infection presents a confounding factor. As Dr. Finkel indicates, O.A.S.’s breathing could have improved because she was no longer infected with this virus. Dr. Finkel’s view appears to be in accord with O.A.S.’s treating pediatrician, Dr. Gavin, who stated “[t]hey went ahead and gave her a 4-day course of IVIG, but it is questionable as to what degree she responded.” Pet’r Exhibit C at 188.

Moreover, after the second and third doses of IVIG, O.A.S. improved only slightly, if at all. If O.A.S. truly suffered from an immune-mediated neurological disease like GBS or CIDP, then she probably would have made a more significant improvement. Thus, on the whole, O.A.S.’s lack of response to IVIG tends to favor SMARD, but only slightly.

## **11. Treating Doctors**

As the signs and symptoms described above arose, O.A.S.’s treating doctors assessed her condition. Their opinions, in many circumstances, may be “quite probative.” See Capizzano, 440 F.3d at 1326.

Two factors limit the value of some opinions from treating doctors. First, there is a question of nomenclature. Second, there is a question about whether O.A.S.’s treating doctors knew about SMARD before 2003.

The first issue concerns the words, such as “peripheral neuropathy,” some doctors used to label O.A.S.’s condition. The Simanskis appear to interpret this phrase as supporting their claim that O.A.S. suffered GBS/CIDP. See Pet’r

Posthr'g Br. at 20. However, the phrase “peripheral neuropathy” – without any modification – does not distinguish SMARD from GBS/CIDP. SMARD also affects the peripheral nerves. Tr. 758 (Dr. Maertens: SMARD “does affect peripheral nerve[s]”), 884-88, 993. In contrast, the phrase “post-infectious demyelinating neuropathy,” Pet'r Exhibit E at 1975, points to GBS/CIDP because those conditions are demyelinating conditions and SMARD is not.

The second concern is historical. Dr. Maertens and Dr. Finkel agreed that most pediatric neurologists were not aware of SMARD until 2003, when an article was published in *Annals of Neurology*. Tr. 756-57, 882-83, 906; see also exhibit S (Grohmann 2003). Dr. Maertens testified that he first learned about SMARD in 2005 or 2006. Tr. 803.

This lack of awareness needs to be taken into account when evaluating statements such as Dr. Rubin's interpretation of O.A.S.'s first EMG. Dr. Rubin indicated that this EMG did not present evidence of a disease of the anterior horn cell. Pet'r Exhibit E at 1953.

Dr. Finkel agreed with Dr. Rubin that the February 26, 2001 EMG did not show evidence of a disease of the anterior horn cell. He also conceded that SMARD is classically classified as a disease of the anterior horn cell. Tr. 1076. However, Dr. Finkel explained that even with today's knowledge of SMARD, evidence of SMARD is difficult to find. He added that O.A.S.'s results alone were not sufficient to eliminate the existence of an anterior horn cell disease. Tr. 1075-78.

In the context of discussing a different EMG, Dr. Finkel explained how EMGs and nerve conduction studies can, in a way, mislead the pediatric neurologists into missing SMARD. Because a nerve conduction study directly measures the peripheral part of the nerve, a doctor interpreting an EMG may focus on the apparent problem with the peripheral nerve and not infer that there is also a problem in the motor neuron process. Tr. 993-94. Dr. Finkel maintained that “[n]one of these people can be faulted for not identifying a motor neuron problem because you've got this peripheral process that's hitting you in the face, and it's profound. So it's very hard to find something subtle that's upstream when everything is downstream because that's what you're measuring directly on the EMG and nerve conduction.” Id.

These caveats affect the assessment of the statements of treating doctors. The Simanskis have identified some statements that support their position that

O.A.S. suffered from GBS/CIDP. See Pet'r Posthr'g Br. at 20-21. Examples include:

- March 7, 2001, Dr. Kotagal stated, "The prolonged distal latency in the [left] peroneal is suggestive of a demyelinating process, an acute or subacute inflammatory demyelinating neuropathy." Pet'r Exhibit E at 2151.
- On March 8, 2001, a progress note indicated, "Given the [increased] phrenic nerve latency[,] considering Guillain Barre syndrome [with] primarily axial and phrenic nerve involvement." Pet'r Exhibit E at 2152.
- A March 8, 2001 record also stated, "[Question of] AIDP variant." Pet'r Exhibit E at 2153.
- On March 17, 2001, her discharge diagnosis was "probable post-infectious demyelinating neuropathy." Pet'r Exhibit E at 1975.

All these statements originated with doctors at the Mayo Clinic during O.A.S.'s first stay there. After O.A.S. returned to Mercy, went home, and returned to Mercy again, her intensivist, Dr. Napa, sent her to Johns Hopkins because the "lack of a diagnosis has been a problem in addressing the extent of supporting the child." Pet'r Exhibit D at 334-36.

At Johns Hopkins, Dr. Crawford noted "a provisional diagnosis of infantile GBS was made." Pet'r Exhibit F at 2349-50. When she was discharged to University of Iowa, her discharge diagnosis was "inflammatory and toxic neuropathies." Exhibit 4 at 28.

An extensive discussion of other statements from treating doctors that are cited in the Simanskis brief is unnecessary because the opinions of O.A.S.'s doctors changed in 2003. In that year, Dr. Kuntz indicated that O.A.S.'s condition "is compatible with a recently described entity called spinal muscular atrophy with respiratory distress or SMARD." Exhibit 1, Vol. 3, at 1481. Again, there is no dispute that most pediatric neurologists did not know about SMARD until 2003. Thus, it is significant that in the year that SMARD became widely known to the relevant medical community, O.A.S.'s doctor at the Mayo Clinic brought up the possibility of SMARD. Tr. 759, 906.

O.A.S.’s treating physicians have consistently referenced SMARD as the proper diagnosis since 2003. See exhibit 1, Vol. 3, at 1457 (Dr. Gavin’s November 11, 2003 letter stating that O.A.S. has “a currently working diagnosis of SMARD”); Id. at 1192 (at admission to Mercy in February 2004 it was noted “[k]nown neuromuscular disorder – SMA-RD type”); exhibit 20 at 1 (a letter dated January 11, 2007 from Dr. Kabbani noted that O.A.S. is “a 6-year-old girl with a clinical diagnosis of sensorimotor axonal neuropathy that also can be called spinomuscular atrophy with respiratory distress”); exhibit 19 at 107 (a letter dated October 8, 2008 from Dr. Flores that O.A.S. “has Spinal Muscular Atrophy with Respiratory Distress”); Tr. 1000.<sup>46</sup>

The Simanskis have done very little to refute these conclusions. At best, they argue that the repetition of diagnosis is an “echo” from the original diagnosis of Dr. Kuntz. Pet’r Posthr’g Reply at 5 n.6; accord Tr. 762-63 (Dr. Maertens); but cf. Tr. 1000 (Dr. Finkel: other people followed Dr. Kuntz’s diagnosis because she is an authority in the field). Other than point to the lack of confirmation via a genetic test, the Simanskis have not offered any argument to say why Dr. Kuntz’s conclusion is wrong. See Tr. 758-59.

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<sup>46</sup> In their reply brief, the Simanskis identified a single post-2003 medical record in which Dr. Gavin described O.A.S. as “a four-year-old female with Peripheral Neuropathy of unknown etiology. . . . One medical consultant has suggested she may have Spinal Muscular Atrophy with Respiratory Distress but this diagnosis has yet to be confirmed.” Pet’ Reply Br. at 9 (quoting exhibit 1, Vol. 2 at 780).

The need for definitive confirmation is discussed in the section regarding Dr. Maertens and Dr. Finkel.

## 12. Comparison between Dr. Maertens and Dr. Finkel<sup>47</sup>

The final important evidence is the testimony from the doctors retained to testify in this litigation. Although both Dr. Maertens and Dr. Finkel possess the minimal training and experience to opine about SMARD, GBS, and CIDP, Dr. Finkel's qualifications were relatively stronger.

Like Dr. Maertens, Dr. Finkel is board-certified in pediatrics and neurology with a special competence in child neurology. Tr. 864. Unlike Dr. Maertens, Dr. Finkel also possesses separate board certification in electrodiagnostic medicine and a separate subspecialty certification in neuromuscular medicine. Exhibit O (curriculum vitae) at 2; Tr. 864.

Dr. Finkel's training and experience in electrodiagnostic medicine enable him to perform EMGs and nerve conduction studies. He also typically reviews the data of tests performed by other people and offers his interpretation of the study. Tr. 865. With this background, Dr. Finkel made a very helpful presentation about how EMGs and nerve conduction studies are done. See Tr. 940-54; Resp't Trial Exhibit D. Consistent with how he evaluates his patients, Dr. Finkel also reviewed O.A.S.'s EMGs and nerve conduction studies.

Dr. Finkel has written more than 60 articles published in peer-reviewed journals. Almost all of Dr. Finkel's articles discuss pediatric neuromuscular issues. He has also served on the editorial board of the journal, *Neuromuscular Disorders*. Exhibit O at 10-17; Tr. 869-70.

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<sup>47</sup> As noted in footnote 15 above, the parties rely upon the opinions of their neurologists to establish O.A.S.'s diagnosis. The parties presented very brief testimony from the two immunologists. Like Dr. Finkel, Dr. McCusker stated that O.A.S. suffered from SMARD. Tr. 431-32.

Dr. Shoenfeld stated that, in his opinion, O.A.S. did not suffer from a genetic disease because "I would expect that if there is a genetic disease affecting the neurons and the muscle eventually that she will be born, as usually is written here, with defects, and that did not happen in her case." Tr. 151. Dr. McCusker explained that many genetic disorders are not manifest until later in life. Tr. 432-34. When asked about Dr. Shoenfeld's opinion that the genetic disease would be apparent at birth, Dr. Finkel stated "[t]hat is the most bizarre comment I've heard in a while." Tr. 1187-88.

Dr. Finkel's focus on neuromuscular disorders fits quite tightly with the issue of whether O.A.S. suffered from SMARD or GBS/CIPD.<sup>48</sup> His opinions regarding O.A.S.'s condition were consistent and persuasive.

On the other hand, Dr. Maertens's opinions lacked consistency and persuasiveness. The primary place where Dr. Maertens seemed to change his position was with regard to signs and symptoms that either were "consistent" or "inconsistent" with the diagnosis of SMARD. In his report, Dr. Maertens argued that certain signs and symptoms, such as O.A.S.'s IUGR and the onset of her neuromuscular disease when she was less than three months old, were not consistent with the SMARD diagnosis. Exhibit 37 at 1-2. However, his testimony on cross-examination revealed that he used the term "consistent" in an unusual way, roughly synonymous with "diagnostic for" the condition. See Tr. 768. When Dr. Maertens was asked to state whether particular signs and symptoms were "consistent" with a SMARD diagnosis, using the more conventional understanding of consistent, Dr. Maertens generally agreed that the sign or symptom was consistent with SMARD. See Tr. 765-66 (Dr. Maertens recognizing that IUGR is consistent with SMARD), 777 (Dr. Maertens recognizing that GBS is extremely rare in children O.A.S.'s age).

When Dr. Maertens was asked to identify the signs or symptoms that most supported his opinion that O.A.S. suffered from GBS/CIDP, Dr. Maertens relied upon two features. First, that O.A.S. was healthy before she suffered respiratory arrest associated with her RSV infection. Second, that O.A.S. responded well to IVIG. See Tr. 860-61.<sup>49</sup> For the reasons explained above in section IV.B.2. and 10., neither of these factors weighs strongly in favor of GBS/CIDP.

In rejecting SMARD as a diagnosis, Dr. Maertens referred to the lack of a "definitive test," Tr. 687, apparently meaning a genetic test. Tr. 819-20; accord Tr.

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<sup>48</sup> Dr. Finkel belongs to groups advocating for treatment for patients with spinal muscular atrophy. Tr. 866-87. However, these groups do not necessarily promote research for SMARD. See Tr. 1177-78.

<sup>49</sup> In this passage, Dr. Maertens also asserts that O.A.S. developed a second autoimmune disease, Henoch-Schönlein purpura. However, neither party discussed the significance of this disorder in their briefs.

672 (Dr. Maertens: no “conclusive evidence for SMARD”). Dr. Maertens’s search for a genetic test is consistent with how he treats his patients. Tr. 820-22; see also Tr. 697. And if O.A.S. were his patient, he would recommend a genetic test. Tr. 822.<sup>50</sup>

However, it seems illogical to point to the absence of genetic testing when the Simanskis could have obtained the genetic testing. Dr. Maertens’s desire for definitive results from a genetic test would seem to elevate the burden of proving the diagnosis to a level above the preponderance of the evidence standard.

### **C. Synopsis**

As noted previously, special masters are required to evaluate the record as a whole. 42 U.S.C. § 300aa—13. Here, the weight of the entire record indicates that O.A.S. suffers from SMARD. Specific factors include:

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<sup>50</sup> Dr. Shoenfeld, too, wanted a genetic test for O.A.S. He stated “if there is still suspicion [that O.A.S. suffers from SMARD], I would say that the Court should have ordered a gene analysis of her case. . . . If the gene analysis . . . will pinpoint toward this diagnosis, I will bend forward, but if not, there is no evidence that this is a diagnosis.” Tr. 161-62.

GBS/CIDP			Data Point	SMARD		
Consistent	Neutral	Inconsistent		Inconsistent	Neutral	Consistent
	•		<b>IUGR</b>			•
•			<b>Health before Respiratory Arrest</b> (some dispute whether O.A.S. had a weak cry)			• (weakly)
		• (extremely rare)	<b>Age of Onset</b>			• (strongly)
		• (strongly – absent reflexes are diagnostic criteria)	<b>Reflex and Weakness Pattern</b>			•
		• (but not impossible)	<b>Permanent Ventilator Support</b>			• (strongly)
		• (strongly)	<b>Sural Nerve Biopsy Showing No Demyelination</b>			•
		• (but not impossible)	<b>CSF Protein – Normal</b>			•
	•		<b>Creatinine Levels</b> (results varied; a question about Mayo results)			• (weakly)
		• (weakly – better response to 2d and 3d dose is expected)	<b>Response to IVIG</b>		•	
		• (strongly)	<b>EMG and Nerve Conduction Studies</b> (showing axonal damage, not demyelination)			• (strongly)

O.A.S.'s course is very much consistent with SMARD. In contrast, as Dr. Finkel noted, O.A.S. would be an exception in almost every diagnostic criteria for GBS. Tr. 1220. Dr. Maertens's opinion that O.A.S. suffered from GBS, which relapsed and turned into CIDP, largely ignores or minimizes the significance of most of O.A.S.'s symptoms. See Tr. 811-15 (Dr. Maertens repeatedly stating that various data points do not support either diagnosis). Under these circumstances, the preponderance of evidence supports the finding that O.A.S. suffers from SMARD.<sup>51</sup>

## V. Causation

The finding that O.A.S. suffers from SMARD is dispositive of whether the Simanskis are entitled to compensation. SMARD is caused by a genetic mutation. Exhibit P (Eckart) at e148, exhibit Q (Giannini) at 1851.

Although the Simanskis attempted to establish, by a preponderance of the evidence, that O.A.S. did not suffer from this genetic based disorder, the evidence convincingly shows that her disease is SMARD. The Simanskis have not presented any alternative theory for how O.A.S.'s two-month vaccinations could have caused (or aggravated) SMARD. Despite having an opportunity to review the reports of Dr. Finkel and Dr. McCusker, neither Dr. Shoenfeld nor Dr. Maertens attempted to link O.A.S.'s vaccinations with her SMARD in their reports. Similarly, the Simanskis elicited no testimony on direct examination from either Dr. Shoenfeld or Dr. Maertens about O.A.S.'s vaccinations having played a causal

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<sup>51</sup> Relying upon a medical article, the Simanskis argue that various signs and symptoms are not useful in predicting whether a person will test positive for the genetic mutation associated with SMARD. Pet'r Posthr'g Reply at 8. There are various problems with this argument. First, it appears to be an attorney argument as the Simanskis cite no testimony concerning this point. Second, the argument is presented only in their reply brief. Given that the Secretary and Dr. Finkel have maintained that O.A.S.'s signs and symptoms were sufficient to diagnose her with SMARD, even in the absence of genetic testing, the Simanskis could have and should have raised this point much earlier in the proceedings. Finally, the Simanskis' approach is inconsistent with the practice of their expert, Dr. Maertens, who has diagnosed his patients with SMARD in the absence of genetic testing. Tr. 698.

(or aggravating) role under the assumption that O.A.S. has SMARD.<sup>52</sup> The Simanskis' post-trial brief pursued compensation based upon only GBS/CIDP.

Under these circumstances, there is no need to explore in detail whether the vaccines could have adversely affected O.A.S.'s SMARD via the Althen test. As the Federal Circuit has explained, “[i]n the absence of a showing of the very existence of any specific injury of which the petitioner complains, the question of causation is not reached.” Lombardi, 656 F.3d at 1353.

The Simanskis have not met their burden of establishing that a vaccine caused or aggravated the disease from which O.A.S. suffers. Consequently, they are not entitled to compensation.

## **VI. Conclusion**

A preponderance of the evidence establishes that O.A.S. suffers from spinal muscular atrophy with respiratory distress, not Guillain-Barré syndrome or chronic inflammatory demyelinating polyneuropathy. The Simanskis have offered no evidence to establish that O.A.S.'s vaccinations caused her to suffer SMARD. Therefore, the Simanskis are not entitled to compensation.

The Clerk's Office is directed to enter judgment in favor of respondent unless a motion for review is filed.

**IT IS SO ORDERED.**

s/Christian J. Moran  
Christian J. Moran  
Special Master

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<sup>52</sup> Although the Simanskis did not introduce the topic, in two instances, Dr. Shoenfeld and Dr. Maertens opined about a connection between vaccination and SMARD. On cross-examination, the Secretary's attorney asked Dr. Shoenfeld whether O.A.S.'s vaccines could have caused or aggravated SMARD. Dr. Shoenfeld replied that it was “very conceivable.” Tr. 164. He did not present any evidence to support this possibility. Tr. 164-65. Dr. Maertens offered a similar opinion and, likewise, did not identify any basis for his conjecture. Tr. 817-19.