Common Drug Review
Patient Group Input Submissions

elosulfase alfa (Vimizim) for Mucopolysaccharidosis IVA (Morquio A syndrome, or MPS IVA)

Patient group input submissions were received from the following patient groups. Those with permission to post are included in this document.
The Canadian Society for Mucopolysaccharide & Related Diseases Inc. — permission granted to post.
The Isaac Foundation for MPS Treatment and Research — permission granted to post.

CADTH received patient group input for this review on or before August 28, 2014

CADTH posts all patient input submissions to the Common Drug Review received on or after February 1, 2014 for which permission has been given by the submitter. This includes patient input received from individual patients and caregivers as part of that pilot project.

The views expressed in each submission are those of the submitting organization or individual; not necessarily the views of CADTH or of other organizations. While CADTH formats the patient input submissions for posting, it does not edit the content of the submissions.

CADTH does use reasonable care to prevent disclosure of personal information in posted material; however, it is ultimately the submitter's responsibility to ensure no personal information is included in the submission. The name of the submitting patient group and all conflict of interest information are included in the posted patient group submission; however, the name of the author, including the name of an individual patient or caregiver submitting the patient input, are not posted.

The Canadian Society for Mucopolysaccharide & Related Diseases Inc.

1. General Information

Name of the drug CADTH is reviewing and indication(s) of interest	Vimizim (elosulfase alfa)
Name of the patient group	The Canadian Society for Mucopolysaccharide & Related Diseases Inc.
Name of the primary contact for this submission:	[redacted]
Position or title with patient group	[redacted]
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Permission is granted to post this submission	☒ Yes ☐ No

1.1 Submitting Organization

Founded in 1984, The Canadian Society for Mucopolysaccharide and Related Diseases Inc. (The Canadian MPS Society) is committed to providing support to individuals and families affected with MPS and related diseases, educating medical professionals and the general public about MPS, and raising funds for research. The Society is a nationally registered charity governed by its Board of Directors with the support and expertise of its Medical Advisory Board. The Society has approximately 125 paid members for 2014, most of whom are affected families.

1.2 Conflict of Interest Declarations

a) We have the following declaration(s) of conflict of interest in respect of corporate members and joint working, sponsorship, or funding arrangements:

We receive unrestricted grants and event sponsorships from the following companies: Genzyme Canada (a Sanofi Company), Shire Canada, BioMarin Pharmaceutical.

b) We have the following declaration(s) of conflict of interest in respect of those playing a significant role in compiling this submission:

N/A

2. Condition and Current Therapy Information

2.1 Information Gathering

The information to complete Section 2 was gathered by conducting:

- telephone interviews with six affected individuals/carers who participated in the Phase III clinical trial of Vimizim ERT
- an electronic survey of Canadian affected individuals/carers living with MPS IV A
- in-person conversations during a regional patient meetings & a national family conference

2.2 Impact of Condition on Patients

Mucopolysaccharidosis IV A (MPS IV A, also known as Morquio A syndrome) is a devastating and complex disease. As you will be aware from reading the literature and clinical trial information, symptoms include hernias, chronic ear infections, hearing impairment, corneal clouding, diarrhea, heart disease, respiratory disease, sleep apnea, hyper-flexibility, dysostosis multiplex (bone disease), spinal stenosis often leading to spinal cord compression, and short stature.

As there has been no treatment for the root cause of MPS IV A (an enzyme deficiency) until the advent of enzyme replacement therapy (Vimizim, or elsulfase alfa), treatment until now has been symptomatic and has only been able to mitigate the progression in these various organ systems. Hernia repair, hearing aids, corrective lenses, CPAP (continuous positive airway pressure) and BiPAP (bi-level positive airway pressure) are common interventions. And although surgical intervention is ubiquitous in those with MPS IV A (100% of respondents have had some type of orthopedic surgery, and some have had up to six surgeries – including knee stapling, hip replacement, spinal fusion and spinal cord decompression), acute pain persists and of those surveyed, all responded that pain – particularly in the spine, hips and knees – has a huge negative impact on the quality of life of for them (when affected individuals responded) or their affected children (when the responses came from caregivers). Clearly enormous amounts of pain, together with hyper-flexibility, skeletal dysplasia, respiratory disease and short stature, lead to immense challenges with mobility, and mobility issues affect day-to-day life and quality of life more than any other factor.

Those surveyed commented that due to their symptoms, they have difficulty with self-care (for many, it's impossible to reach the back of their heads), holding objects, and doing things like opening doors due to decreased wrist strength. They are reliant on others for assistance. One mother of two affected children said, "They just can't do what an average person does. Even taking a shower takes 45 minutes and is exhausting. Never mind going for a walk – just putting on their shoes to do that is a huge challenge. They don't have the dexterity to tie their laces and watching them try to get up off the floor can be heartbreaking."

All respondents have difficulty walking long distances and climbing stairs, which means they are often excluded from sporting, school and/or social activities with their peers or even family members (some reported that they have to avoid certain relatives' homes as they cannot manage to get up and down their stairs). Due to social isolation and the stigma associated with using wheelchairs, those affected reported challenges with self-esteem and difficulty in forming relationships. All respondents have had to give up activities that they enjoyed before the symptoms of MPS IV A increased to the point that they were no longer able to participate: some of the children loved to play baseball but cannot catch a ball anymore; some used to love playing mini-stick hockey, but cannot get up off the floor to play anymore; some enjoyed dance classes until they could not keep up; others have given up riding their bikes (one

mother commented that her 11-year-old son loved riding his bike so much that when he could no longer manage that on his own, he tried with training wheels, but eventually had to give it up altogether. He also had to give up swimming, even though he had previously been on his school's swim team); and some have given up cooking and baking as it's become too difficult to reach shelves or lift pans. Even preparing simple snacks is impossible for some of the individuals surveyed.

2.3 Patients' Experiences With Current Therapy

As mentioned above, patients are currently managing their disease symptomatically. Hernia repair, ear tubes, tonsillectomies & adenoidectomies, hearing aids, corrective lenses, CPAP and BiPAP are common interventions. Respiratory medications are used by some, pain medication is used universally, and surgical intervention is ubiquitous in those with MPS IV A (as mentioned above, knee stapling, hip replacement, spinal fusion and spinal cord decompression are common procedures). Despite these interventions, symptoms persist and worsen. One mother likened surgical interventions to "band-aid" fixes as they often have to be repeated.

All respondents surveyed commented that post-surgery recovery time caused hardships for not only those convalescing, but on all family members. Time off school was cited as creating challenges for patients, and time off work caused financial stressors for caregivers.

Respondents also cited delays in surgery scheduling and challenges accessing rehabilitative care, including physical therapy and occupational therapy.

Most respondents said that CPAP and/or biPAP help them manage their sleep apnea, at least to a certain extent; however, most responded that their respiratory disease continued to progress, with their breathing laborious.

As mentioned previously, extreme and persistent pain was cited by all respondents, and no current therapy was managing this pain adequately. One parent commented that her daughter's joint pain "isn't even touched" by the pain medications she takes. She also suffers from stomach upset when she takes them. While acupuncture may help some patients, it's expensive and not easily accessible.

2.4 Impact on Caregivers

All respondents to our survey cited missed work, difficulty finding suitable work or the inability to work full-time as a major stressor. Every single person surveyed reported financial, emotional and relationship stress. Some of the financial stress came from costly home renovations and costly devices and equipment, but much of it came from the employment challenges previously mentioned. Parents commented that their caregiving roles are emotionally and physically draining, and that they are always "on". One mother compared caring for her adolescent daughter to caring for a toddler, as constant attention is required. One mother commented that she is always "waiting for the phone to ring" when her child is at school and that she has suffered from panic attacks during and following her son's surgeries due to worries about the high anesthesia risk for those with MPS IV A. Parents also cited that the demands of navigating the medical, educational and social services systems are exhausting. The extra layer of complexity when planning, scheduling (and executing) their and their children's lives is extremely time-consuming. The impact on siblings was also reported across the board, with many parents feeling their unaffected children's lives are affected by the overall adaptations and sacrifices required due to MPS IV A in the family.

These day-to-day challenges are exacerbated during surgical and convalescent times, with surgical recovery sometimes taking weeks or even months.

3. Information about the Drug Being Reviewed

3.1 Information Gathering

The information to complete Section 3 was gathered by conducting:

- telephone interviews with six affected individuals/carers who participated in the Phase III clinical trial of Vimizim ERT
- an electronic survey of Canadian affected individuals/carers living with MPS IV A
- in-person conversations during a regional patient meetings & a national family conference

3.2 What Are the Expectations for the New Drug or What Experiences Have Patients Had With the New Drug?

a) Based on no experience using the drug:

Those patients/caregivers who responded to our survey expect that Vimizim will help stabilize the progression of their/their children's disease. As mobility was cited as a huge factor in quality of life (QoL), it is expected that an increase in mobility (due to a stabilization of the skeletal, joint, respiratory symptoms) will significantly increase the patients' QoL. An increase in growth was also cited as a major potential benefit of treatment, since untreated, some patients may only reach 90 cms. In addition, potential benefit relating to alleviating risk of cervical cord compression is seen as meeting a critical unmet need.

All respondents said they would be willing to accept serious adverse events in order to experience the benefits. In addition, all respondents commented that they are willing, and even happy, to spend a day a week, even if travel is required, receiving a scheduled enzyme replacement therapy infusion in order to stabilize their/their children's disease, recognizing that stabilization would mean less hospital time for other medical procedures/surgeries/recovery time in hospital.

b) Based on patients' experiences with the new drug as part of a clinical trial or through a manufacturer's compassionate supply:

Of all surveyed, no respondents currently receiving Vimizim reported any negative effects or adverse effects from treatment. Although some respondents cited the time to travel to/from hospital for weekly infusions as onerous, they were happy to put in the time to receive the benefits of treatment. All commented that they/their children tolerated the infusions well, with no pain or discomfort, and actually enjoyed the time they spend with the medical teams at the trial centres, as they have found enormous emotional support and "hope" through the process of being involved in the treatment.

The benefits of treatment reported include increases in weight, strength, height (some have grown several cms. during the past several months), and overall energy levels. Those surveyed said their breathing is better (one mom said her daughter "no longer has to gasp for breath"), their snoring has decreased (one young man no longer requires biPAP overnight) and ear and upper respiratory infections have decreased substantially. Those on treatment report waking up feeling better rested, being able to get through the day with a more positive attitude and without naps (which previously were common). Mobility, which is as previously mentioned the most important indicator of quality of life in those with MPS IV A, has increased dramatically while on treatment. The young boy who had given up swimming can now swim back and forth across the pool while his mom watches proudly. One young woman on

treatment can walk double the length she could when she started the trial. Stair climbing has gotten easier. One girl on the trial could previously only walk up 2-3 stairs and generally had to be carried. Now, she can manage 30-40 stairs at a time. Several respondents commented that they can now go places they previously couldn't, like long grocery stores aisles and relatives' homes that were previously inaccessible. One young woman who drives said her increased mobility means she doesn't always need to transport her wheelchair in and out of her vehicle as she can now manage short errands without a mobility aid. This has had a huge impact in her ability to lead a more normal, independent life.

Overall, those on treatment have seen their disease symptoms stabilize and they overwhelmingly report better mental health due to knowing their disease is no longer taking its natural course. They are doing something about it. They have hope for a better future.

The Isaac Foundation for MPS Treatment and Research

1. General Information

Name of the drug CADTH is reviewing and indication(s) of interest	VIMIZIM – (Elosulfase alfa) – MPS IVA -Morquio A Syndrome
Name of the patient group	The Isaac Foundation for MPS Treatment and Research
Name of the primary contact for this submission:	[redacted]
Position or title with patient group	[redacted]
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Telephone number(s)	[redacted]
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Patient group's contact information:	
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Telephone	[redacted]
Address	[redacted]
Website	http://www.theisaacfoundation.com http://www.morquio.ca
Permission is granted to post this submission	☑ Yes ☐ No

1.1 Submitting Organization

The Isaac Foundation's mission is to fund innovative research projects that aim to find a cure for MPS, a rare, debilitating, and progressive disease. We provide support for families of individuals suffering from MPS and advocate on their behalf to ensure government funding for expensive, life-sustaining treatments are covered by the health care system.

1.2 Conflict of Interest Declarations

a) We have the following declaration(s) of conflict of interest in respect of corporate members and joint working, sponsorship, or funding arrangements:

1. **BIOMARIN PHARMACEUTICALS:** The Isaac Foundation has received sponsorship grants from Biomarin Pharmaceuticals over the course of the past 5 years. Sponsorship has been granted to our Annual GALA FOR A CURE, an event designed to raise funds to find a cure for MPS VI. Biomarin has been a sponsor of this event since its inception 5 years ago.

2. **SHIRE PHARMACEUTICALS:** Our organization has received sponsorship funds from Shire Pharmaceuticals for various events over the past year. These sponsorship grants go to our MPS II Research program, with all dollars raised going directly toward research projects that aim to find a cure for MPS II (Hunter Syndrome).

3. **JANSSEN PHARMACEUTICALS:** Janssen Pharmaceuticals, a division of Johnson and Johnson, began supporting our GALA FOR A CURE in 2013 and has committed to supporting our event again in 2014. All money provided by Janssen goes directly toward funding research projects aimed at finding a cure for MPS.

b) We have the following declaration(s) of conflict of interest in respect of those playing a significant role in compiling this submission:

The Isaac Foundation has no conflict of interest to declare with respect to anyone playing a significant role in compiling this submission. All work, interviews, and drafting of this submission was supported and completed by our organization alone.

2. Condition and Current Therapy Information

2.1 Information Gathering

Information for Section 2 was obtained using one-to-one conversations with a number of patients using the current therapy, as well as parents of patients on therapy. Personal information was also used, as our organization helps and supports numerous families suffering from Morquio A Syndrome. We also have referenced printed sources, published articles, available clinical trial data, and discussed the condition and experiences using the new therapy with the lead investigator responsible for the VIMIZIM clinical trial here in Canada.

2.2 Impact of Condition on Patients

Morquio A Syndrome is a disease that has numerous life-altering and very progressive symptoms. These symptoms include but are not limited to bone and joint disease, heart and airway disease, progressive stiffening of the joints, corneal clouding, hearing loss, and decreased endurance.

All patients that were interviewed for this submission reported that Morquio A Syndrome affects all aspects of their lives. One patient noted, ***"Morquio affects all parts of my life. Every movement has to be thought about to try to minimize pain. From rolling over in bed, to standing up at the kitchen sink to getting into a vehicle all requires forethought."***

A parent of two affected individuals expressed similar experiences dealing with the disease: ***"They are very tiny, which creates a world of issues all in itself, but the amount of energy required to do everyday things is remarkable and exhausting. They cannot do what an average person can do in a day without becoming very tired and sore. The fatigue they feel in their legs and joints is incredibly painful and not always manageable with pain medication. They require regular rest times and time to just find the energy to finish their day. Even just getting dressed is exhausting and doing something as simple as walking up our stairs in our two story home requires a lot of effort."***

"My joints are all affected and because of this I have progressively had to rely on a wheelchair for mobility. Obviously there are limits associated with using a wheelchair that I must deal with. Even grocery shopping is a difficult thing when you have to consider how to carry (the groceries) to the van in the snow in a wheelchair." – Patient Interview

Morquio A Syndrome is a very progressive disease, and all patients suffering from the condition that were interviewed reported numerous activities that they could not do that used to be a "normal" part of their life. Examples of lost abilities include bike riding, ice skating, walking on a beach or trail, dressing themselves, and grocery shopping.

The aspects of this condition that are most important to control are endurance and bone and joint disease. These two symptoms seem to play a crucial role in the quality of life of affected individuals. Improved endurance has an impact on all facets of life and endurance is a good measurement on how well a patient's body is performing. From heart to bones to pulmonary function, increased endurance indicates that those systems are working better. With respect to bone and joint disease, the stabilizing or improving of this aspect of the disease would lead to a better quality of life, reduced reliance of mobility devices, and the potential for pain reduction in all affected joints.

All patients and caregivers interviewed expressed a desire to see the disease stabilized or the progression of the disease slowed down or halted. ***"The most important thing from a mom's perspective is the slowing of the progression of this disease. We are told from diagnosis the disease is progressive and degenerative until the eventual death of our children. There is now hope with the prognosis of slowing this progression and giving our children a more productive and a better quality of life."***

2.3 Patients' Experiences With Current Therapy

Before Vimizim was approved by Health Canada, patients suffering from Morquio A Syndrome have had no access to treatment (unless they took part in the clinical trials). Prior to Vimizim, treating the disease was done by managing symptoms as they appeared. This has led affected individuals to undergo numerous surgical interventions, with more severe effects being seen as the disease progressed. In Canada, 21 patients have participated in the clinical trial, and the patients that I interviewed have all seen dramatic improvements in their condition and general quality of life.

One caregiver noted the impact she has seen on her children while participating in the clinical trial for Vimizim: ***"Since starting on Vimizim the boys have certainly had much more energy and endurance for doing everyday things. They are taking much less pain medications and have had a noticeable decrease in the amount of corrective orthopaedic surgeries. Their lung function tests are improved and the amount of times the boys have had lung related issues has been almost non-existent since starting this trial."***

A consistent theme that showed in all interviews was how stable patients were since undergoing treatment with Vimizim. All reported a marked stabilizing of the condition, which is remarkable for such a progressive disease. One patient remarked what stability really meant to him, and why such stability was important to his quality of life: ***"I think with determination and Vimizim – I would say that I am mostly stable and somewhat improved in some areas. Some might think that "Stable" is not a big thing, but for me it is HUGE. To understand what that means to me you have to understand my situation a bit better. In the period leading up to being invited to participate in the study – I had noticed a steady decrease. Being in my 30's I was getting old for Morquio. It is degenerative. I had noticed the wear and tear on my joints beginning to show. I had a steady increase of pain in my joints and more problems were what I needed to expect. I went to doctors about the problems as they began to show up – things like my elbow, my shoulder, and the breakdown of my neck fusion. Each looked at my x-rays or MRI and saw how far the disease had progressed and did not want to proceed***

unless the pain became unbearable – the concern was that they could make it worse and if a bone shattered I might lose everything." The symptoms he discusses have been stabilized and no medical interventions were needed.

Current treatment with Vimizim is very effective in controlling aspects of this disease. Recent clinical trial data made available by Biomarin Pharmaceuticals showed a dramatic increase in endurance for patients participating in the clinical trial. This increase in endurance has the potential to manifest improvements into all aspects of disease progression. Endurance is a good indicator on how well the body is working, and any such increase works to improve overall quality of life.

In addition, patients have reported a noticeable drop in medical interventions since beginning treatment through the clinical trial. One caregiver reported that her son had been a candidate for a major operation on his knee and leg bones, a surgery that was expected due to the progression of the disease over a number of years. After some time on the clinical trial, the orthopaedic surgeon now believes that the surgery can be pushed into the future, or may not be needed at all. While he was not able to conclude that Vimizim was the reason that the long-planned surgery didn't need to take place, he did note that the only thing to change for the patient was his participation in the clinical trial. He noted significant stabilization in the knee and leg bones and expressed his belief to the caregiver that the enzyme replacement therapy was the reason.

"We have certainly had so many positive aspects it's really hard to find any negative aspects to this therapy. The possibility of the boys have a better quality of life and living longer is really all we hope for and everything else is a bonus." – Caregiver Interview

However, treatment using enzyme replacement therapy (ERT) for this condition does not come without some level of hardships. While none of the patients interviewed experienced any adverse reactions during their infusions or afterward, Biomarin Pharmaceuticals does indicate on their labeling that anaphylaxis, or a severe life threatening reaction, can occur during the infusion process.

Primarily, the hardships reported during interviews centered on location and travel time associated with receiving infusions. Many patients have to travel multiple hours to and from infusion sites, which makes the process take the better part of one or two days. Added travel to an already tiring infusion process creates an exhaustion level for patients that is difficult to recover from. All patients and their caregivers express a keen desire to have home-infusions. This will cut travel time for infusions down to zero, allows families to better schedule their lives around infusion timelines, and allow patients and caregivers the ability to lead a more normal life. Caregivers expressed a desire to return to the work force full time, and patients expressed a desire to miss less schooling. Home infusions would allow that for all involved.

2.4 Impact on Caregivers

Caregivers face significant challenges caring for patients with Morquio A Syndrome. First and foremost, the stress managing a loved one's progressive disease is unquantifiable, and as many supports as possible should be put in place to help all caregivers of patients suffering from Morquio A Syndrome.

Patients suffering from Morquio A Syndrome require significant medical interventions, long hospital stays, many surgical procedures, and repeated appointments with a host of specialists. These visits,

operations, and appointments cannot be done alone, and caregivers sacrifice a lot of their own time to ensure patients receive company and support during these visits.

While such sacrifice from caregivers is difficult to imagine, it doesn't come without its own rewards for them. One mother expressed both the challenges and the reward in her patient interview: ***"There have always been hardships in having children affected with this disease. The many surgeries, the financial strain of finding equipment for the boys' mobility, the home renovations and van conversions, the emotional stress of having children with so many medical needs has been hard on our family but have also proven to bring us all closer together as a family."***
Many caregivers must leave the workplace for all or a portion of the workweek in order to care for their loved ones battling this disease. With ERT infusions, this may still be the case due to the current need to infuse in a hospital setting. This leaves parents and partners of those affected bringing their loved ones into infusion centres for the day or two-day long treatment (depending on travel time).

None of the caregivers or patients interviewed have suffered any adverse effects related to therapy with Vimizim and thus could not comment on what challenges, if any, they would have if such adverse effects were to be experienced.

3. Information about the Drug Being Reviewed

3.1 Information Gathering

Information for Section 2 was obtained using one-to-one conversations with a number of patients using the current therapy, as well as parents of patients on therapy. Personal information was also used, as our organization helps and supports numerous families suffering from Morquio A Syndrome. We also have referenced printed sources, published articles, available clinical trial data, and discussed the condition and experiences using the new therapy with the lead investigator responsible for the VIMIZIM clinical trial here in Canada.

3.2 What Are the Expectations for the New Drug or What Experiences Have Patients Had With the New Drug?

a) Based on no experience using the drug:
It is expected that the lives of patients will be improved significantly by this new drug. First and foremost, and much like other enzyme replacement therapies being used for MPS related diseases, stabilization of the disease is expected to occur in all individuals, regardless of when treatment begins. Clinical trial data also points to increased endurance and a decrease in overall GAG accumulation in the urine of Morquio A Syndrome patients. The decrease in this GAG accumulation indicates a lower storage of these GAGs in the bones, tissue, organs, and muscles of patients. Accumulation of these GAGs results in the clinical symptoms that individuals experience and a reduction in the body would indicate a slowing-down of the disease progression.

Currently, there are no other treatments available for patients so there is a tremendous unmet need for this ERT. The adverse effects that were experienced in the clinical trial setting were very minor compared to the benefit that this treatment offered patients. The opportunity to see improvement in endurance, bone and joint disease, and heart and pulmonary function far outweighs the relatively minor allergic reaction that can sometimes occur during the infusion process. In addition, such reactions during infusion are rare and easily managed by slowing down the infusion process or halting it until the reaction has been corrected and maintained.

Any improvement in this condition can have a profound effect on the quality of life for patients suffering from this disease. All such improvements lead to fewer hospital visits, fewer medical interventions, and fewer doctors' appointments. The results of that lead to less time off work, less time away from school, and more time together as a family. All of these benefits are unquantifiable from a socio-economic perspective, but are incredibly important to the well-being of patients and their families.

b) Based on patients' experiences with the new drug as part of a clinical trial:
All of the patients that were interviewed and on the clinical trial for Vimizim reported improvements in endurance and stabilization in their condition since they began therapy. There were no comments regarding negative effects of the drug on their conditions. One patient summed up the following about their experience on this new drug***: "Vimizim has helped me have more energy and has kept my condition stable over the past 3 years. I certainly look forward to being able to live my life in much better shape than I expected before I started Vimizim because at that time I had no help, no treatment. Now that I am on Vimizim, when I have a minor setback, I feel confident that it will level out again. It has given me hope that my condition will continue to be stable with my current lifestyle for years to come."***

All of the reported adverse effects were thought to be acceptable for patients receiving this therapy, especially since this is the only treatment available for Morquio A Syndrome.

"The drug has changed my outlook on long-term health a lot, I used to think I had an expiry date – one that in my 30's I was getting closer and closer to. Now I don't think of the disease that way – more something that I have to deal with on a day-by-day basis. Overall it has had a truly positive effect on my well-being." – Patient Interview

The impact of this treatment on the patients and families battling Morquio A Syndrome cannot be understated. All patients discussed the improved quality of life they have experienced since treatment began for them. Older patients on the clinical trial stated their wish was to have had access to this therapy earlier in life, so that some symptoms could have been diminished or avoided altogether. In addition, caregivers expressed the joy and hope that this treatment provides their family, while others talked about the future more than they would have prior to beginning treatment. This was a common theme amongst interviewees, and is summed up nicely by this caregiver: ***"We are hopeful now with this new therapy that we can enjoy many more years together. We all have a new found hope for the future."***

4. Additional Information

The questions included in this template were very clear and helpful. It would be helpful to families and patients if they could provide direct input to your review team, rather than having to direct that input through an organization. We appreciate your time and consideration of this document and look forward to watching Vimizim work its way through the CDR process in a timely fashion.