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CADTH COMMON DRUG REVIEW

Patient Input

CERLIPONASE ALFA (BRINEURA)

Biomarin Pharmaceutical (Canada) Inc.
Indication: Neuronal Ceroid Lipofuscinosis Type 2

CADTH received patient input from:
Individual patient/caregiver

November 8, 2018

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Patient Input Template for CADTH CDR and pCODR Programs

Name of the Drug and Indication	cerliponase alfa (Brineura)
Author of the Submission	The following is the experience of a Canadian family with a child with Batten Disease CLN2, whose physician provided the family with CADTH's contact information for the purpose of providing input to this review.

1. About Your Patient Group

[Not applicable]

2. Information Gathering

Personal experience with cerliponase alfa (Brineura)

3. Disease Experience

Our [redacted]son was diagnosed with Batten Disease CLN2 October 2016.

His initial symptoms were seizures (initially diagnosed as epilepsy). He was given medication which at the time, appeared to halt his epilepsy. After 3 years seizure free, he was weaned from the medication. (*Note: he has remained seizure free from that time onward - but is not typical of other children with Batten Disease- although there are a few other children who also do not experience seizures, most do experience seizures)

Before beginning treatment with this medication, the disease had a profound and quick progression on his body and intellect. The symptoms seemed to descend upon him at about 8 years old.

When he was 6-7 years old he was given an Educational assessment by an Educational Psychologist (the assessment was actually gifted to us by his grandparent, who had also gifted Educational Assesssments to all of the children in our extended family at that age). The assessment results indicated that the only issue he appeared to have was dyslexia (this is/was prevalent in our family, as both my husband and I struggled with dyslexia in our youth).

Physically, his symptoms started with his gait - he appeared to have trouble walking and running. Soon after he began to fall onto the floor while walking or standing.

At the same time that his gait issues appeared, (8 yrs) we were approached by his teachers who noticed significant changes in his cognitive abilities. We sent him for another Educational Assessment and that one indicated that he had a significant intellectual disability. He went from having dyslexia to a significant intellectual disability in a 2 year period.

After many tests with his Pediatric Neurologist, he was finally diagnosed.

From the time he was 8, to the beginning of his treatments (approximately a 2 year period) we watched our beautiful boy deteriorate before our eyes. He went from being a star little league baseball player, to being unable to walk to first base.

He went from being able to walk freely without issue, to using a walker full time, then onto using a wheelchair while at school (he still uses a walker at home, but only with full assistance).

The play structure that he used daily, swinging, climbing and running around, stopped being used because he couldn't even make his way outside to use it.

He went from being able to use a pencil to write, to being barely able to write his own name. Teachers would teach him a concept and the next week he would remember none of it. His speech became harder and harder to understand.

We were told to prepare ourselves for the rapid, agonizing death of our only child who one day would no longer be able to talk, swallow, eat, sit up or see. Our once bright, beautiful boy was going to wither and die before our eyes.

[redacted], we need for you to understand that the ONLY hope we had to save our boy was to get him access to cerliponase-alfa. I know with 100% certainty, without that medication, my son would be a shell of the boy he is today.

Once he started treatment, his rapid decline ceased and plateaued. We are fully aware that this medication will not repare the skills he has already lost. What it does do however, is stop or significantly slow down further deterioration from occuring.

So, to answer your question about how Battens disease impacts patients' and caregivers' day-to-day life and quality of life, I can tell you that today we are at a place where we are helping our boy to live his life despite the damage Battens already did to him. He has difficulty coordinating movements (ataxia). He can still walk with a walker and additional assistance. He has difficulty speaking. His speech can sound mumbled, but his teachers, family and friends can understand him. He struggles with an intellectual disability. It takes him longer than average to understand concepts, but he engages in adult conversations.. and he has the most amazing sense of humour!!

I know this may sound odd, but with Batten kids there is one very beautiful aspect to this disease - these kids remain such beautifully sweet, innocent kids. I'd say my almost 13 year old boy has the wonder and innocence of an 8 year old. That can be such a beautiful thing to see in this day and age.

When you ask me to describe how the disease impacts patients' and caregivers' day-to-day life and quality of life, I can tell you that the damage to him BEFORE receiving treatment means that today I quit my full time job and became his caregiver. He absolutely needs a caregiver wirh him to help him with transfers, bathing, food preparation, safety measures. He travels in his wheelchair outside of home, so transportation requirements are arranged for him to/from school (he is in a special needs classroom and has an Individual Education Plan IEP).

To answer the part if you question asking if there are any aspects of the illness that are more important to control than others - honestly, now that he receives treatment, the answer is no.. aside from keeping up his Physiotherapy and Occupational Therapy to maintain his strength and dexterity. Honestly, now that he has treatment, we are just dealing with the fallout of what the disease did BEFORE he started receiving treatment. I know of many families in the United States where there are 2 children- the first one was diagnosed after symptoms started ravaging their bodies- those kids, like ours, live their lives with the damage done, but no longer progressing... the siblings of those children got tested before symptoms appeared- were diagnosed before any sign of Battens began rearing it's ugly head - and today those children receive treatment and remain symptom free (despite reaching and exceeding the age where the disease 'should' be showing up). Sadly, for some of those families they were too late.. their children were too far gone - the disease had done too much damage.. their children were/are g-tube fed, suctioned daily, unable to see, speak, move.. and the families made the heart wrenching decision to not begin treatment, allowing the disease to swallow their children.

4. Experiences With Currently Available Treatments

[redacted], this is currently the ONLY medication available to children with CLN2. Without it, my child would be at a very different stage of the disease than he is.. of that, I have no doubt.

Honestly, any difficulties that would present themselves to accessing the medication would have no impact at all on our decision to attain it. Like most parents, we would do anything to save our boy's life. Honestly, the only difficulty we experienced was getting the treatment into Canada. Before that, we travelled every 2 weeks to Nationwide Childrens Hospital in Columbus, Ohio. We sold our house to be able to afford to do that. I quit my job and we went down to one income to be able to afford to get him access.. and I would do it all over again in a heartbeat. Because the thing with Batten Disease is that every single day without medication makes a HUGE impact on the child's life.. each day lost means more brain cells die - more damage done. This disease is that incidious. I know we are so fortunate to have access at SickKids Hospital (one hour from our home).. it rips a hole in my heart knowing somehow my son got access in Canada, when other children and families wait without theirs. There was finally another child granted access (in Winnipeg) but there are more waiting. Imagine how it would feel knowing there is a drug available that could keep your beautiful child alive, and that many children worldwide were getting access to... but not your child.. you could do NOTHING because some beurocrats aren't sure if it's worth it to agree to it... I just can't fathom how there is even a decision to be made.. why can't they just grant access to those children.. they hold those children's life in their hands. What if it were your child? What if your child was developing completely normally then all of a sudden had this disease overtake them.. how would you feel if your child's doctor told you they were going to die, but that there was one medication available.. oh, but you couldn't give it to them because you live in

Canada and it's not approved here yet. There are no words to convey that type of helpless horror. I know these parents. They are my friends.

Every 2 weeks we go to SickKids Hospital where my son is given a wellness check then the pharmacy is notified to remove and prepare the med (it's frozen and needs to thaw). He is given the pill formats of Tylenol, Benedryl and Ondansetron and numbing cream is put upon the skin covering port in his head. A Neurosurgeon accesses the port and withdraws CSF for lab testing. Once thawed, his medication is given via infusion directly into his port. Following the med, there are 2 rounds of flush given to ensure no medication remains in the tubing. The process takes approx 4.5 hours once the medication starts to flow. We typically spend a full day at the Hospital from 8am-4:30ish - the extra time accounts for the time it takes for his wellness check, waiting the 1.5hrs for the med to thaw, waiting on the Neurosurgeon to come access etc.

He tolerates the med very well - eating, drinking and socializing throughout the entire infusion.

Other than fatigue caused by Benedryl and Ondansetron, there are no side effects or issues encountered on the day of the infusion.

The day following his infusion, we choose to keep our son at home - mostly because he remains somewhat tired from the full day at hospital. Other than some fatigue, he experiences no other side effects.

5. Improved Outcomes

This question doesn't quite apply to us.. the only thing better I guess, would be gene replacement therapy.. but that isn't even available at this point.

6. Experience With Drug Under Review

Again, this doesn't quite apply. Besides Cerliponase alfa, there is nothing else available. Is it working .. YES!! We know that because we bore witness to the steep, frighteningly fast decline of our son before his treatment started.. and we have since witnessed the halting of that decline.

7. Anything Else?

No. I think I said everything I need to above.

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Appendix: Patient Group Conflict of Interest Declaration

To maintain the objectivity and credibility of the CADTH CDR and pCODR programs, all participants in the drug review processes must disclose any real, potential, or perceived conflicts of interest. This Patient Group Conflict of Interest Declaration is required for participation. Declarations made do not negate or preclude the use of the patient group input. CADTH may contact your group with further questions, as needed.

1. Did you receive help from outside your patient group to complete this submission? If yes, please detail the help and who provided it.

   No

2. Did you receive help from outside your patient group to collect or analyze data used in this submission? If yes, please detail the help and who provided it.

   No

3. List any companies or organizations that have provided your group with financial payment over the past two years AND who may have direct or indirect interest in the drug under review.

Company	Check Appropriate Dollar Range			
	$0 to 5,000	$5,001 to 10,000	$10,001 to 50,000	In Excess of $50,000

I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this patient group with a company, organization, or entity that may place this patient group in a real, potential, or perceived conflict of interest situation.

Name:
Position:
Patient Group: The individual who submitted this input has declared no conflict of interest.
Date: