Dear Mr. Trudeau, Ford, McColeman, Bouma and Ms, Hajdu, Elliott

I am writing to request your assistance on behalf of Marcellinus K Muljarahardja to help

Hid health condition, Marcell who have been diagnosed with a very rare and debilitating disease,

to receive a life altering treatment. Marcellinus K Muljarahardja have been diagnosed with Spinal Muscular Atrophy (SMA). They are in desperate need of a lifesaving gene therapy called “Zolgensma” that comes at a staggering cost of $2.8 million. There is no way we could afford this without your intervention. Please help us.

Spinal Muscular Atrophy (SMA) is the genetic disease that affects the central nervous system,

peripheral nervous system, and voluntary muscle movement. SMA affects a child’s ability to sit

independently, crawl, stand, and walk. SMA can also affect one’s ability to speak. Left untreated,

a child may develop difficulty breathing, swallowing, and even die.

Few beautiful babies in Ontario, Including Marcellinus K Muljarahardja (Ontario-Brantford), have SMA Type 2.

Stephanie and Tiffany Chhuon (Ontario-Maple), another twins girl, was also recently diagnosed with SMA.

On December 16, 2020, Health Canada approved Zolgensma. The provinces are still in the process of negotiating funding which we understand will take time. However, time is not something that we have the luxury of given that Zolgensma should be administered before the age of two and / or prior to certain limited weight (under 21kg) in order to be effective and minimize permanent damage to the children. For Stephanie and Tiffany (the twins) turned 2 years old in September 2020.

Currently, Spinraza is the only available treatment in Canada. It has to be administered via a

painful lumber puncture every 4 months for a **lifetime**. Spinraza costs approximately $700,000

for the first year and the continued cost of it will be around $360,000 per year for the rest of their

lives. This treatment, however, only helps to slow the progression of SMA. It is not a cure. In

the long run, the cost of Spinraza far exceeds Zolgensma, which is the only treatment with the

promise to provide better a quality life for a child. The Spinraza treatment process is a constant

source of strain and anxiety for the families and these procedures are incredibly traumatic for the children.

Zolgensma works to replace the broken gene that causes SMA and it is amazing to see that a

child with this one-time treatment would become like a normal child in all the aspects, said by

Dr. Reiger, President and CEO of the Canadian Organization for Rare Disorder. Dr. Wong-

Reiger also indicated that “By the time Health Canada and the provinces approve the funding for this therapy, which we believe inevitably will happen, it’s going to be too late for some of these kids because of that two-year requirement”. These children have struggled so much with their disease, not to mention the painful Spinraza treatment that absent your support, they will have to endure for a lifetime.

Marcellinus K Muljarahardja neurologist has indicated that they are waiting for the funding for Zolgensma. Marcellinus and the twins girl have already received 4 treatments of Spinraza which is delivered

via painful lumbar puncture and has significant side effects. We have not seen an improvement

resulting from this treatment. Given the poor results to date and the prejudice that the passage of time is having on their conditions, I beg you to please consider intervening in this matter to help us afford the Zolgensma treatment.

With every day that goes by, their neuron motor function degrades. They do not have time to wait. As

parents we are begging you to please intervene to help Marcellinus, Stephanie and Tiffany, to

obtain these one-time lifesaving doses of Zolgensma prior to it being funded in Ontario.

Furthermore, we hope Ontario government will help funding Zolgensma without putting any limitation.

We thank you kindly for your consideration.

Best Regards,

Daniel Muljarahardja

