July 8, 2020

AN OPEN LETTER TO CANADA'S PROVINCIAL MINISTER OF HEALTH

A fortuitous confluence…serendipitous happenstance…alignment of stars. When the very best strategy for a patient is also the most cost-effective for the governments, there should be no hesitancy to act. Especially when the need for action is urgent.

Good News. Across Canada there are a handful of babies with Spinal Muscular Atrophy Type 1 who have the good fortunate of being born when there is a funded therapy that can stop the most devastating impacts of a disease that destroys motor neurons and robs them of the ability to eat, breathe, and sit up. Even though Spinraza requires a spinal infusion four times a year, the therapy keeps the infants alive and significantly improves functioning.

Very Good News. For the 50 (approximately) SMA-1 babies who will be born in Canada each year in the future, they will be blessed to be diagnosed immediately through newborn screening (hopefully) and to have immediate access to the gene therapy, Zolgensma. It is a one-time administration will stop the disease before motor neuron damage occurs. And the babies develop normally.

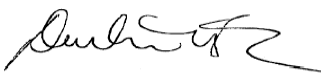
Challenging News. Today, across Canada, there are a handful of SMA-1 babies under two years if age who were lucky to have had access to Spinraza from the time of diagnosis of a few weeks to several months. But the single-infusion Zolgensma is not yet approved and funded in Canada and by the time the process is completed, these children will be "too old" to qualify for the drug which has to be administered before the age of two and before there is significant damage.

Kaysen, Wyatt, Eva, Nathan, Lucy. Babies hoping to get Zolgensma as soon as possible. Right now, their only chance is Novartis' global managed access program (aka "the lottery"), which every two weeks randomly selects two babies from a worldwide pool of applicants to receive a compassionate treatment. Health Canada recognizes their requests as urgent, having granted each of them approval to the gene therapy through the federal government's life-saving Special Access Programme. But they still need funding.

We are appealing to the provincial governments to fund Zolgensma for these "in-between" infants even as the drug is undergoing Health Canada and CADTH/INESSS review and in advance of negotiations with the pCPA. We are sure the gene therapy will be funded; not only is it the superior treatment and avoids regular lumbar infusions but the single administration is much more cost-effective over the life of the patient. The comparison of Spinraza and Zolgensma by the independent agency ICER in the USA demonstrates this in five- and ten-year projections.

It is not always possible to have a treatment that is unequivocally superior in outcomes and in quality of life impact but also more cost-effective. This is an extraordinary request but these are extraordinary circumstances. We urge you to act immediately to provide the funding in advance of the regulatory approval and reimbursement negotiations.

Best,



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