



Clinic for Special Children

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May 22, 2020

RE: Siyona Shrestha (DOB 12 September 2019)

Treatment of spinal muscular atrophy (SMA) with Zolgensma™

Onasemnogene abeparvovec (Zolgensma™), is a gene replacement therapy developed to treat the genetic cause of spinal muscular atrophy (SMA). In clinical trials, this one-time treatment has proven effective in improving the lives of the children affected by this life-threatening genetic disorder. The treatment was recently approved by the FDA as a single-dose intravenous (IV) infusion for infants under two years of age who have genetically confirmed SMA and do not have neutralizing antibodies to the treatment.

The Clinic for Special Children and Lancaster General Hospital participated in the last clinical trial of this drug and continue to administer it following FDA approval. However, we are only able to do so for Siyona once her family has secured the drug, which costs \$2.1 million USD. In addition to the cost of Zolgensma™, Siyona's family will also need the funds to pay for the hospitalization and monitoring needed to safely administer Zolgensma™, estimated and outlined in the table below. Siyona will need to remain in the United States and in the care of our clinic for a minimum of 60 days after she receives Zolgensma™ to monitor her for adverse reaction to the therapy. Costs of travel, living expenses, and other medical expenses are not included in this estimate, and would be the responsibility of the family for the duration of their stay.

Zolgensma™ Treatment Estimate Price List			
Item	Price/Unit	Units	Total Price
Consent to Treat	\$150.00	1	\$150.00
Genetic Confirmation	\$0.00	1	\$0.00
AAV9 Antibody	\$0.00	1	\$0.00
Laboratory testing	\$245.00	14	\$3,430.00
Urinalysis	\$17.00	14	\$102.00
Physical Examination	\$70.00	15	\$1,050.00
Motor assessments	\$50.00	5	\$250.00
Glucocorticosteroids (prednisolone)	\$7.00/30mL	TBD	TBD
Zolgensma Infusion	\$736.00	1	\$736.00
Day Medicine Stay	\$137.00/hr	5	\$4,685.00
Total			\$5,718.00