Providing Access to Innovative, Life-Saving Treatment for Children with Spinal Muscular Atrophy: Multidisciplinary Collaboration

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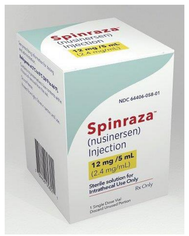
Purpose

- Spinal Muscular Atrophy (SMA) is progressive & fatal.
- In December 2016, the first disease-specific treatment was approved by the FDA.
- The demand for this treatment among UVA patients has been immediate and growing.
- UVA Children's Hospital was the first in the nation to give Nusinersen (Spinraza) therapy in clinical practice following FDA approval.
- The approval of this treatment significantly increased inquiries with 20+ new patients in a few months from around the country.
- Spinraza is delivered intrathecally.



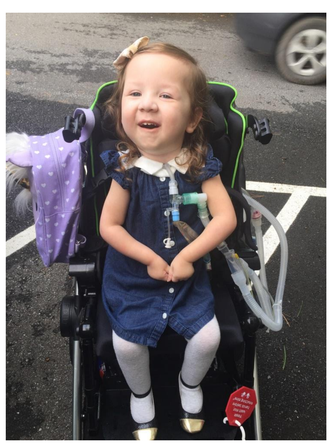
- The aim of this practice-based improvement was to provide rapid, coordinated access to a complicated treatment to children in need.

Significance

- We worked diligently to enable our most vulnerable children, those with Spinal Muscular Atrophy (SMA), gained access to groundbreaking treatment.
- Creativity, education and collaboration were needed to rally the entire health system to support the specific needs of this population.
- UVA has expert teams of physicians, nurses, therapists, pharmacists, nutritionists and other providers who are experienced in the care of SMA.
- Medication delivery and monitoring requires a multidisciplinary team and care coordination.

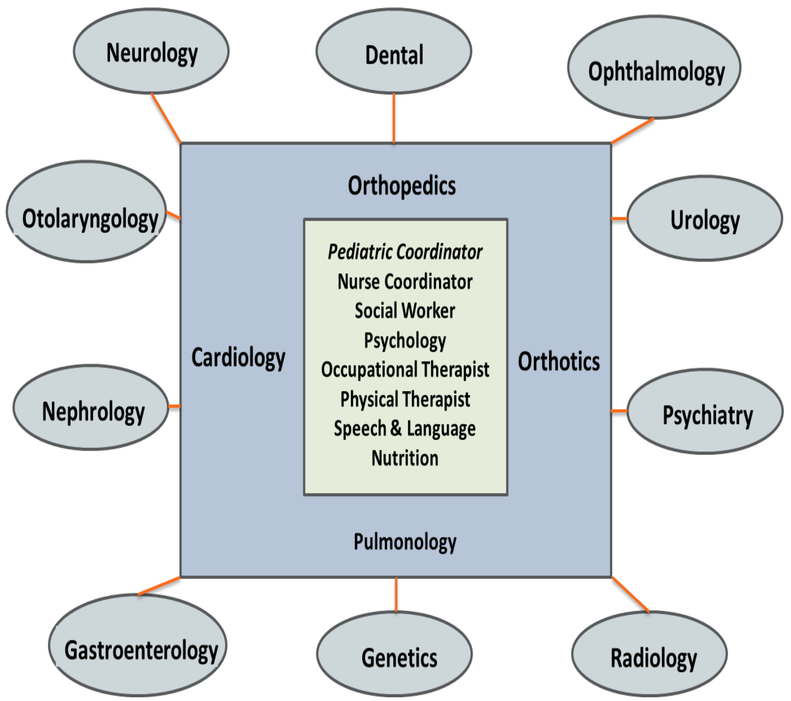
Implementation

- Immediately, a multidisciplinary approach was instated to coordinate intrathecal dosing for medically complex children, largely overseen and coordinated by nursing.
- Treatment with Spinraza required 12 mg intrathecal administration: Loading doses every 2 weeks x 3, plus another loading dose after one month, and then maintenance doses every 4 months continually.
- Prior to dosing, each child evaluated in multidisciplinary clinic by our expert team of physicians, nurse practitioners, nurses, physical and occupational therapists and nutritionists.
- Each child is evaluated using the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND) motor scale.
- Patients are dosed in the operating room, outpatient procedure room or interventional radiology. Location based on medical complexity, sedation and respiratory needs.



Evaluation

- We have dosed 18 children with SMA since February 3, 2017.
- UVA was able to administer the first dose of Spinraza in the country following FDA approval.
- We have administered over 90 doses to pediatric patients.
- Care of children with neuromuscular disorders such as spinal muscular atrophy require a multidisciplinary team and care coordination.
- All UVA patients who receive Spinraza are scheduled to return to our comprehensive clinic every 4 months to monitor their response to the drug and adjust their management.



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