

SLC6A1 Connect's

THREE-YEAR STRATEGIC PLAN



2021-2023

SLC6A1 Connect

SLC6A1 is a rare neurological condition that causes seizures, severe movement and speech disorders, and intellectual disability in children.

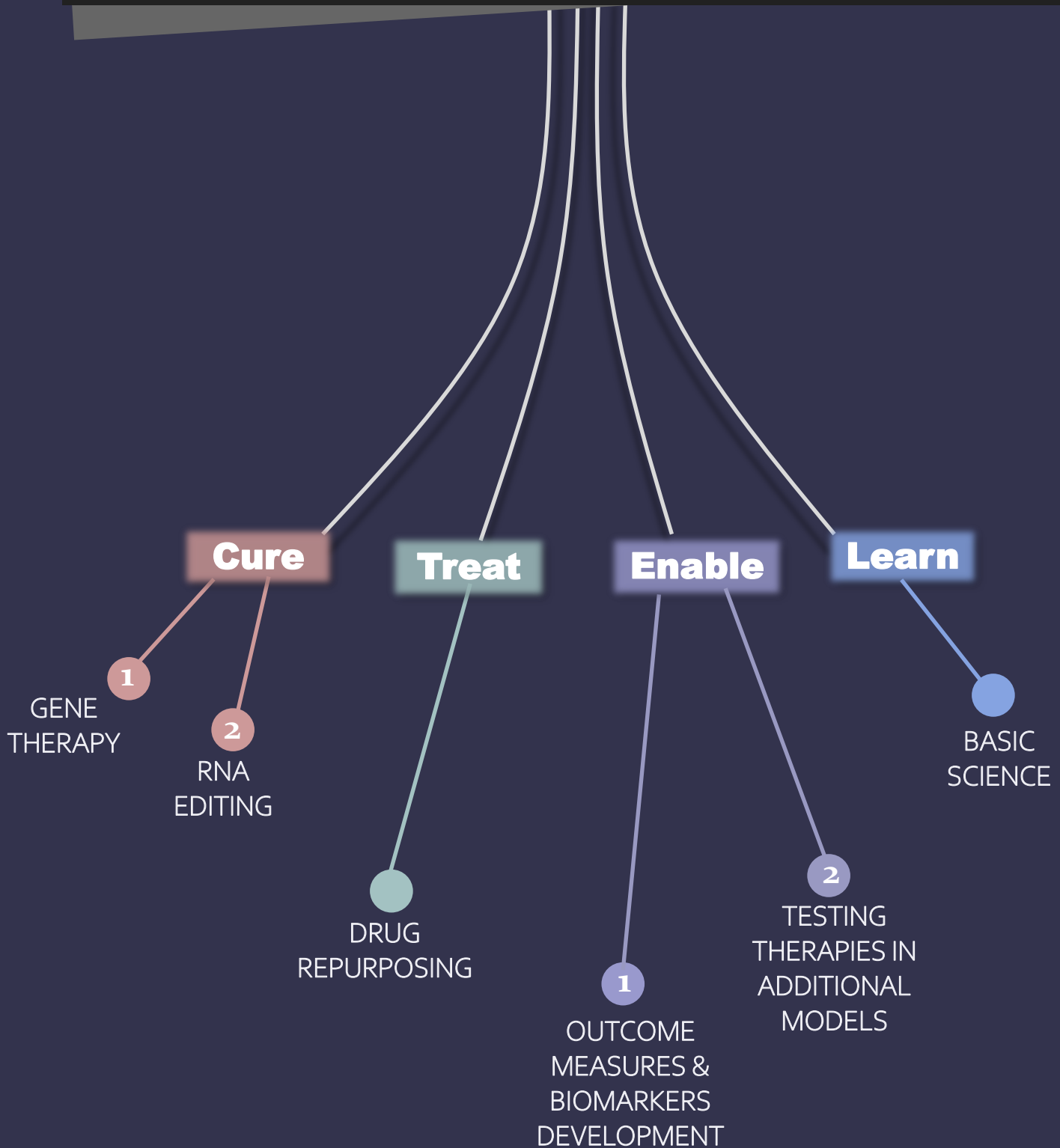
SLC6A1 Connect is a patient advocacy group dedicated to improving the lives of children and families affected by SLC6A1.

Our focus is to raise awareness and fundraising to advance scientific research that will ultimately result in a cure.



*Pepijn, 5 year old
SLC6A1 patient*

The Path to a Cure



1

GENE THERAPY

At the core of the plan are two cutting-edge approaches designed to cure SLC6A1 by attacking the root cause of the disorder: the SLC6A1 gene. Pursued in parallel, these approaches are applicable to all SLC6A1 mutations and deletions.

Of the two approaches, gene therapy is the most advanced and is our lead program. The concept behind gene therapy is simple: deliver healthy copies of the SLC6A1 gene to compensate for the mutated ones. Over the past two years, the progress in developing a gene therapy has exceeded our expectations. We expect improvements, at least to some degree, regardless of age.



*Alora, 9 year old
SLC6A1 patient*

Launch a HUMAN CLINICAL TRIAL

Contingent on sufficient funding and requisite FDA approvals, we plan to begin the **first clinical trial** of gene therapy for people with SLC6A1. Ideally, a biotech or pharmaceutical company will license the program and conduct the trial.

2nd GENERATION PROGRAMS

Technological advances in gene therapy are happening quickly with more effective vectors being discovered that can carry larger DNA cargos and target a greater percentage of brain cells. While we anticipate encouraging results with our first clinical trial there will undoubtedly be room to improve. We will therefore support second-generation gene therapy programs to **leverage all technological advances**.

2

RNA EDITING

Patients with SLC6A1 can't make enough SLC6A1 to transport the body's most important inhibitory neurotransmitter, GABA, because they have a mutation on the gene. This is where RNA Editing can help.

SLC6A1 GENE



RNA TOOL



INCREASED
GABA
TRANSPORT

The technologies to develop oligonucleotide therapeutics differ from traditional drugs by targeting disease at the level of RNA. They have recently become the **first drugs ever approved** to treat the causes of some fatal diseases, including Duchenne muscular dystrophy and spinal muscular atrophy. More than 100 oligonucleotide therapeutics are now in development for other rare diseases, such as Huntington's Disease and ALS, and also for common conditions including cancer and Alzheimer's disease. Oligonucleotide technologies have accelerated drug development to the point that a customized oligonucleotide treatment was recently developed for one single person with a unique, fatal disease in **under one year**.

3

DRUG REPURPOSING

Drug repurposing is a large industry and has a promising future as it is one of the most active areas in pharmacology in the last decade. Existing approved drugs are constantly being tested in order to be used for new purposes, of which they were not originally intended. **This process circumvents** some of the most expensive drug discovery processes, by reducing the development timeline. Drug repurposing typically decreases **the overall cost** of bringing the drug to market because the safety and pharmacokinetic profiles of the repositioned candidates are already established.



Charlie, 3 year old
SLC6A1 patient

Check this out

SLC6A1 Connect is screening more than **20,000** known FDA compounds currently and advancing one candidate into trial!



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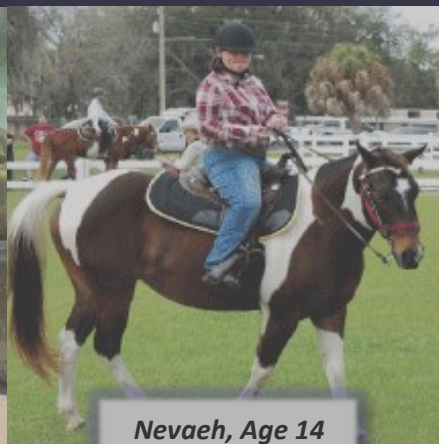
OUTCOME MEASURES & BIOMARKERS DEVELOPMENT

There are currently no FDA-approved outcome measures for use in SLC6A1 clinical trials. We have established a consortium of expert SLC6A1 physicians to develop outcome measures that are meaningful to patients and their families and that are acceptable to the FDA and other international regulatory agencies. SLC6A1 Connect is also a member of COMBINEDBrain, a consortium led by patient advocacy foundations, working with the clinicians, researchers and pharmaceutical firms that are developing treatments for the disorders they represent. One of the aims of COMBINEDBrain is to **develop universal biomarkers** across neurodevelopmental conditions.

SLC6A1 Rockstars



Rowen, Age 3



Nevaeh, Age 14



Xander, Age 3

5

TESTING THERAPIES IN ADDITIONAL MODELS



*Ella, 11 year old
SLC6A1 patient*

Because no animal model can completely duplicate the human disease it is **important to verify results** from animal studies using human cells. Today, technology exists to convert skin or blood cells collected from individuals with SLC6A1 into brain cells. These cells can be used to replicate results observed in animal models. Since brain cells can be generated from any individual with SLC6A1, this technology also allows us to assess whether there are **significant differences among individuals** in response to a new therapy. These models are known as induced pluripotent stem cells or brain organoids.

6

BASIC SCIENCE

Basic science is essential in the development of all translational therapies as we further understand the underlying mechanism of disease. Significant gains in our knowledge about the mutated gene that causes SLC6A1 has played a key role in defining important components of gene therapy and other approaches. **Continuing to expand our understanding** of this gene, its protein product and its function are vital to the success of our research.

SLC6A1 Connect has made numerous basic science grants and continues to make independent small seed grants. Seed grant funding enables scientists to obtain proof of concept and apply for large National Institute of Health grants. SLC6A1 Connect greatly encourages young investigators and under-represented members of the science community to apply through the non-profit directly or through our partners, Uplifting Athletes and the American Epilepsy Society.



Maximize Your Impact

Core Beliefs

Patients First

Every day matters for our children.

Objectivity

“In God we trust, all others must bring data.”
– W. Edwards Deming

Fostering Collaboration

Progress accelerates when people work together and share knowledge.

“Too Rare”

Rare diseases are deemed Too Rare To Care by governments, pharmaceutical companies and academics. **We need you.**

SLC6A1 Facts


- ◇ SLC6A1 is run entirely by mothers trying to **save their children.**
- ◇ Overhead is less than 1%
- ◇ Every dollar raised funds research
- ◇ We are ranked Platinum on Guidestar
- ◇ The Freed family has **personally funded** \$100,000 of research. Amber Freed spends 65 hours a week project managing every scientific endeavor.
- ◇ We have been featured in People Magazine, Huff Post, Bloomberg, CNBC, and BuzzFeed!



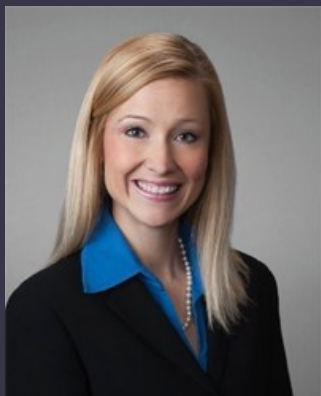
Supporting Programs

SLC6A1 Centers of Excellence

Our participating Institutes offer a comprehensive set of services: not just core services, but advanced and supporting services specifically geared to treating patients with SLC6A1. The range of services often spans the entire continuum of care, not merely the acute care procedure. We currently are working with Children's Hospital of Philadelphia, The University of Texas Southwestern, and Children's Hospital of Colorado to be our first SLC6A1 Centers of Excellence. We will work in the next 3 years to obtain two additional centers- one on the West Coast of the USA and another in Europe to serve and support our International families abroad. The purpose of the SLC6A1 COE Program is to:

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- ◇ Provide superior neurological care to patients in the specific regions of the US and allow **better access for patients** with SLC6A1 mutations to get expert care.
 - ◇ Encourage collaboration between departments, including Psychiatry, Neurology, Developmental Pediatrics, Pediatrics, Occupational Therapy, Physical Therapy, and Physical Medicine.
 - ◇ Provide opportunities to integrate clinical care with teaching and research.
 - ◇ Engage in clinical research to determine **which treatment modalities yield the best results**. The SLC6A1 COE Program shall produce these benefits for the Participating Medical Centers:
 - An interdisciplinary approach to diagnosis and treatment of the neurological problems caused by SLC6A1 mutations
 - An efficient system for treatment of patients dependent upon their symptoms and conditions
 - The provision of both surgical and nonsurgical solutions to patients' problems
 - The objective assessment of different treatment modalities to determine their benefits
 - Efficient use of resources
 - More convenient to accommodate patients involved in clinical trials or pilot programs

Our Founder



Amber N. Freed is fighting like a mother to cure SLC6A1.

Amber Freed is the mother of adorable twins, Miss Riley James and Mr. Maxwell Norman. Maxwell was just 18 months old when the Freed family received his devastating diagnosis of SLC6A1. Ms. Freed left her career in equity analysis the day Maxwell was diagnosed and dedicated her life to finding a cure. In 18 months, Amber has single-handedly driven multiple translational treatments forward and become a leader within the rare disease community.

Ms. Freed serves as the Founder and CEO of SLC6A1 Connect. SLC6A1 Connect's work has elevated awareness and created an ecosystem that can systematically help fund and consolidate research and treatment efforts. Her efforts have been highlighted in the Huffington Post, BuzzFeed, Bloomberg, CNBC and many more.

Ms. Freed was featured in the best-selling book, *Shortcut to Prosperity*, as an example of grit well before her skills were put to the ultimate test. Prior to Founding SLC6A1 Connect, Ms. Freed served in a variety of equity and financial analysis roles, most recently in consumer equity research with Janus Henderson Investors. Prior to Janus, Ms. Freed has served in roles with Stout, Risius & Ross, RK Capital Management, Dividend Capital Trust, and KPMG LLP. Ms. Freed attended the University of Denver for both undergraduate and graduate school, receiving degrees in Accounting on an academic scholarship.

Ms. Freed was nominated for the Global Genes Rare Champion of Hope Award and sits on the Board of CombinedBrain. Amber can be reached at any hour of the day to advance science.

Our Vice President



Kimberly Fry, Vice President of SLC6A1 Connect joined our leadership team in late 2019 after her son, Charles Fry was diagnosed with SLC6A1.

Ms. Fry brings more than 12 years of marketing and event expertise in both higher education and technology industries, currently serving as the Director of Marketing and Events at ProActive Solutions, a technology reseller in Mission, KS.

She has a strong reputation for producing high end, thoughtful, creative and meaningful experiences that lead to awareness and financial support for a wide-range of audiences. Prior to ProActive Solutions Ms. Fry spent her career at the Kansas City University of Medicine and Biosciences, holding many positions, most recently as the Associate Director of University Events and Interim Director of Marketing.

Kimberly attended the University of Missouri-Kansas City, receiving a Bachelors Degree in Business Administration and serving as a four year collegiate athlete as a member of the Universities Division I

Volleyball team.

Kimberly's vision and goal for SLC6A1 Connect is to cure the disease, not continue to treat and live with it. She brings personal passion and determination to give her son, Charles, and all kids affected a chance to thrive and live healthy without the daily worry of this neurological disease.

In addition to Ms. Fry's role at SLC6A1 Connect and ProActive Solutions she is a dedicated mother to son, Charles, daughter, Adeline and wife to Nathan Fry. The family resides in Kansas City, MO.