Background

Prior to 1971, glycogen storage disease type Ia was almost universally fatal marked by extreme failure to thrive, life-threatening hypoglycemia, and acidosis.
Background

In 1982, cornstarch was introduced as a slow release glucose source.
Glycogen Storage Disease in 1998

- Most children in the United States and Canada were getting liver transplants or dying
- No clinical research was occurring
- 16 years without a major advancement

There was little hope for families
Many Children Suffer Due to Lack of Knowledge About GSD
Impact of Treatment
Impact of Treatment
Impact of Treatment

• Avoid excessive intake of carbohydrate
49 States
History

• Program previously located at Boston Children’s Hospital (until 2005) and University of Florida (2005 – 2017).

• Program relocated to Connecticut in January 2017
Rationale for Moving to CT

- GSD families built us clinical and research units at CCMC and UConn

- Joint collaboration between UConn Health and Connecticut Children’s

- This would not have occurred if programs were not connected
UConn GSD Gene Therapy Unit
CCMC GSD Unit

• Located on the 8th floor of the hospital
• 3 bed clinical and research unit created
• GSD program office
• Clinical laboratory
Impact on CT

• Over 200 families have traveled to CT for care and education in the first 10 months

• Over 1000 days of hotels and housing have been required

• 12 families have moved to CT and purchased housed in CT due to the program and this is estimated to increase to over 20 by 2018

• Students coming CT colleges due to the program
Investment in Rare Diseases is Good for the State of Connecticut

- Patients and families will travel for rare disease specialists

- Investment by the state in rare diseases financially benefits the state

- Support for the medical institutions is critical