
THiogenesis THerapeutics

Thiogenesis Therapeutics Corp. (TSXV: TTI / OTCQB: TTIPF)

Next Generation Treatment for Cystinosis and Beyond

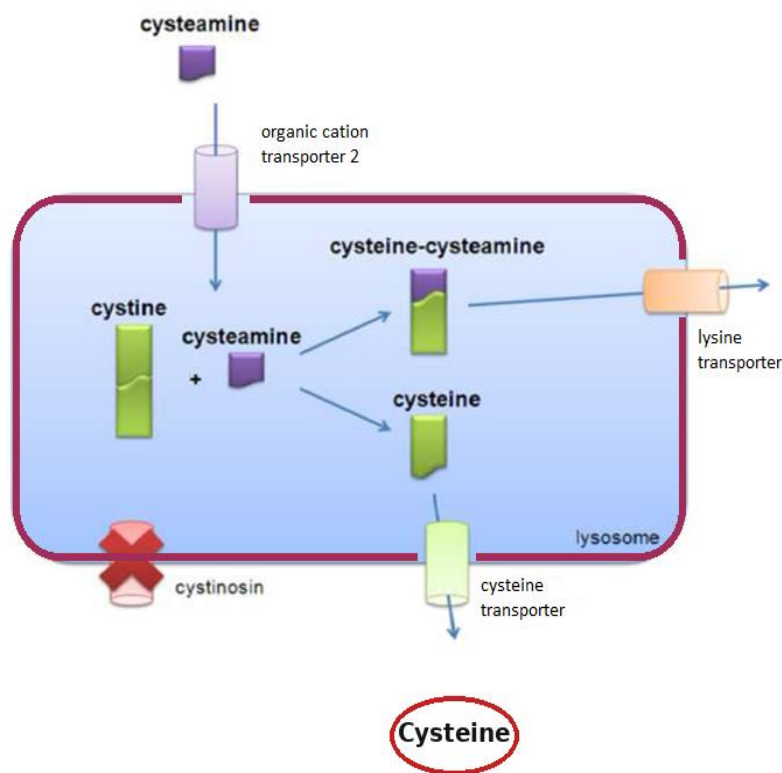
October 2026

Forward-Looking Statement

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Cystinosis: Effective Therapy, Unmet Need



Besouw M — Adapted from et al. *Drug Discovery Today*, 2013

What is Cystinosis

A rare genetic disorder in which toxic cystine builds up inside cells' internal “waste bins” (lysosomes), leading to progressive kidney failure and organ damage in children

Current Treatment Burden

Standard-of-care is cysteamine-based drugs; currently Procysbi® requires patients to swallow **16–28 capsules daily** depending on dose, causing GI distress and pervasive sulfur body odor

The TTI-0102 Advantage

Potential to be a once-daily sachet (powder), eliminating pills entirely — clearing cystine, reducing GI side effects, and providing free cysteine (a precursor to GSH)

Thiogenesis — Overview

Clinical-stage biotech company developing next-generation cysteamine therapies for cystinosis and primary mitochondrial diseases

Proven team

Developed Procysbi® for cystinosis at Raptor (\$800M acquisition)

Next-generation prodrug

TTI-0102 — next-generation, sustained-release cysteamine

Enhanced adherence

Potential to be a once-daily sachet (powder), eliminating pills entirely

Near-term value driver

~\$350M cystinosis market with near-term data and a clear Phase 3 pathway

Platform expansion

Into mitochondrial diseases with significant unmet need

Proven Leadership / Cysteamine Experience

Christopher M. Starr, PhD — Chairman of the Board

Co-founder, BioMarin; co-founder & CEO, Raptor; co-founder, Monopar

Patrice Rioux, MD, PhD — Founder, CEO, Director

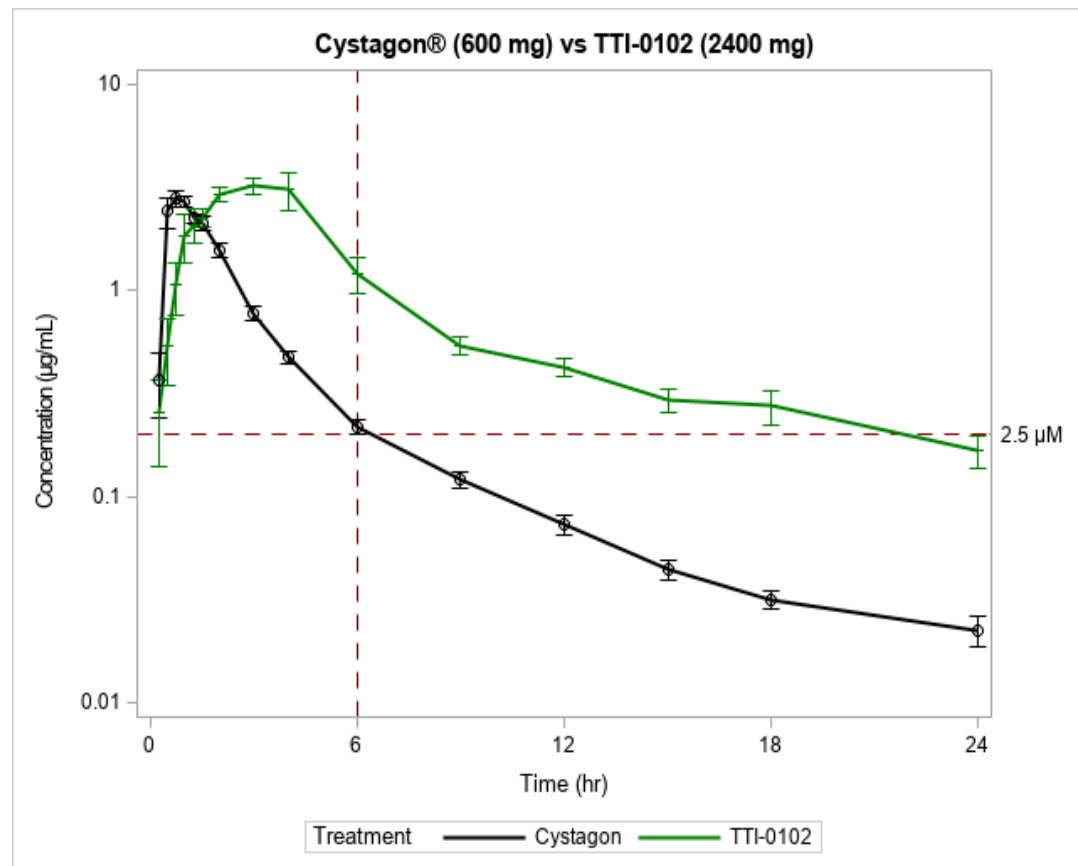
Former Biogen; CMO/Head of Regulatory at Raptor

Deep experience at the intersection of rare disease & redox biology

- Responsible for Procysbi® clinical development, approval, and launch
- Leader in cysteamine biology know-how with decades of clinical use
- Demonstrated ability to build durable value in the public markets

TTI-0102: Human PK and Safety

Evaluated at up to 4× the therapeutic dose



Well-tolerated in healthy volunteers

Up to 4× the therapeutic dose

Mild side-effect profile

Only mild, short-term body odor — no other significant side effects

Sustained drug levels through 24 hours

Supports once-daily dosing

Next step

To be replicated directly in cystinosis patients, with Dr. Greenbaum (Emory)

Adherence Failure Drives Kidney Failure

>90%

Adherence in children under direct parental administration

~70%

Adherence in self-managing adolescents and adults — roughly 3 days in 10 inadequately depleted

Source: Gaillard et al., 2021

G-Tube Solution

For the younger tube-dependent U.S. patients, a soluble sachet entirely replaces the microgranules that routinely cause line damage.

Compounding Organ Damage

Because cysteamine does not reverse established proximal tubular damage, unmanaged days accumulate directly as permanent nephron scarring.

Root Causes of Non-Adherence

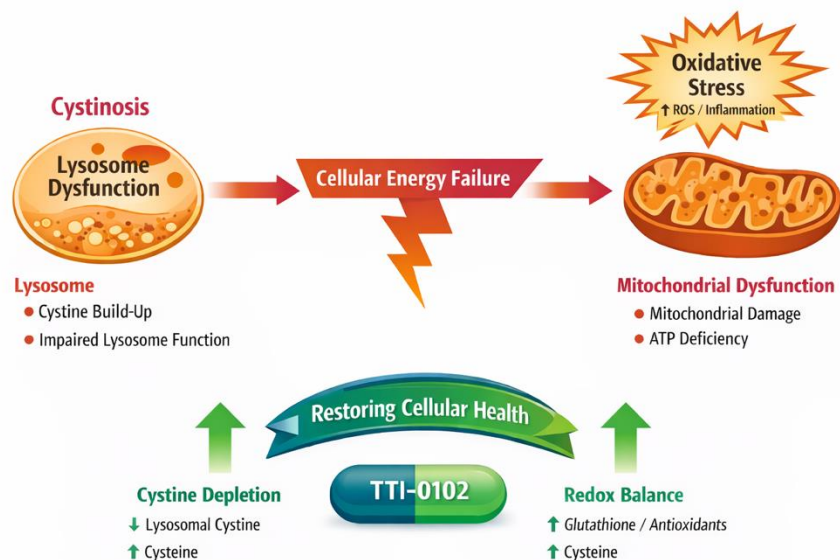
Severe pill fatigue; a rigid 5-hour daily fasting window; constant GI distress; severe social stigma from sulfurous breath and body odor.

A De-Risked Path to Cystinosis Phase 3

Rapid, low-risk path to market

- Senior team brought Procysbi® to market (Clinical & Regulatory, CMC)
- Established cysteamine biology and mechanism of action — approved 2x
- Established clinical endpoints for approval — surrogate biomarker
- Investigator-initiated study with Dr. Greenbaum to optimize Phase 3 design
- **505(b)(2) pathway** → optimized Phase 3 registration study
- Defined and capital-efficient path toward registration in cystinosis

Primary Mitochondrial Diseases



Conceptual schematic adapted from Bellomo & De Rasmo, *Int. J. Mol. Sci.* (2026)

Rare genetic diseases/mutations

Impair the body's ability to efficiently produce cellular energy and release significant free radicals – generating oxidative stress and inflammation

Patient population

Approximately **75,000 patients** affected in the U.S.

Common biochemical features

Low cysteine → reduced glutathione and oxidative stress

Platform potential

Shared disease biology creates potential for a broader mitochondrial disease platform through restoration of **cysteine**

Significant unmet need

Few approved treatment options

Leigh Syndrome

Next step in mitochondrial disease execution

Subgroup of Primary Mitochondrial Disease

Caused by a genetic mutation that impairs the cell's ability to produce ATP

Birth prevalence: ~1 in 40,000 live births

FDA-cleared IND

For a two-stage Phase 2a clinical trial, conducted in collaboration **with a leading children's hospital**

Phase 2a clinical design

Stage 1: randomized, placebo-controlled study (9 patients). Stage 2: pediatric open-label extension (6 patients)

Rare Pediatric Disease Designation

FDA granted RPD designation for Leigh syndrome (July 2026), with eligibility for a Priority Review Voucher upon NDA approval

Near-term catalysts

Trial initiation: Q4-2026. Initial data: Q2-2027

Thiogenesis Development Pipeline

TTI-0102 Indication	Preclinical	Phase 1	Phase 2	Phase 3
Cystinosis (Lysosomal Storage Disease)	✓	✓	Investigator Initiated Study	Next
Leigh Syndrome (Primary Mitochondrial Disease)	✓	✓	2a Planned (Q4-2026)	
MELAS (Primary Mitochondrial Disease)	✓	✓	✓ Complete	
MAFLD (Pediatric) (Metabolic Disease)	✓	✓	Planned	

Summary of Investment Thesis

Near-term value creation, long-term platform opportunity

- Proven cysteamine development team, with a track record of value creation
- Institutions have validated the opportunity
- Leading rare disease KOLs engaged in clinical development
- Established path toward a clearly defined Phase 3 trial in cystinosis
- Cystinosis positioned as the primary near-term value driver
- Leigh offers exposure to a large unmet mitochondrial disease opportunity
- Near-term catalysts capable of supporting a **meaningful valuation re-rating**

Company Info

Shares Issued	69.9 million
Shares Fully Diluted	76.2 million
Insiders (24%)	16.9 million
Share Price (04/09/2026)	\$0.50
52-Week High/Low	\$0.88 / \$0.44
Market Cap.	\$35.0 million
Cash (06/30/2026)	\$7.3 million
Contact	info@thiogenesis.com

Thiogenesis Therapeutics (TSXV: TTI / OTCQB: TTIPF)
Currency in Canadian dollars

Established Market, Durable IP

Commercial Market Dynamics

- \$233M US Procysbi® sales in FY2025 (per Amgen 10-K) at \$300k–\$500k+/patient/year with proven, entrenched reimbursement
- Commercial model focuses on converting fatigued Procysbi® patients and capturing incident pediatric cases at parity pricing
- Europe represents an unpenetrated greenfield market

Intellectual Property Protection

- Composition of Matter: US 11,786,491 B2 and granted EU counterpart (WO2017161318) protect TTI-0102 through 2038
- Method of Use: US 11,612,576 B2 & EP 18858763.8 protect cystinosis and mitochondrial use through September 2037
- Freedom to Operate: Procysbi® regulatory exclusivities expired June 2025; Lupin generic settlement sets licensed entry for March 31, 2030