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

Next Generation Treatment for Cystinosis and Beyond

November 2026

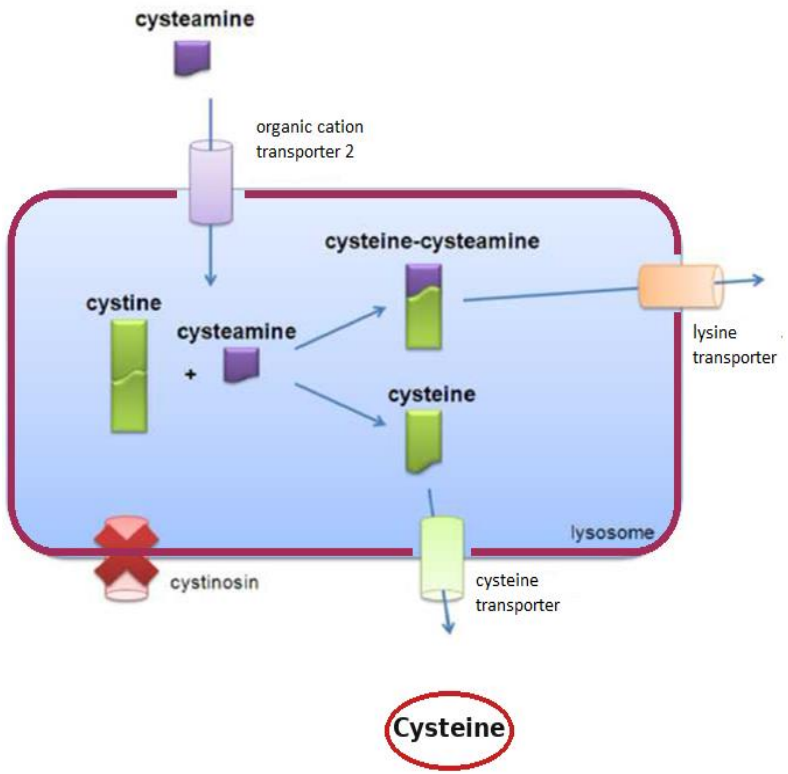
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Cystinosis: Effective Therapy, Unmet Need



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

Target Commercial Format: Once-daily oral sachet (powder), eliminating pills entirely — clearing cystine, reducing peak-driven GI side effects, and restoring free cysteine.

OVERVIEW

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

Target Commercial Format: Once-daily oral sachet (powder), eliminating pills entirely and potentially reducing peak-driven GI side effects

Near-term value driver

~$360M cystinosis market with near-term data and a clear Phase 3 pathway

Platform expansion

Into mitochondrial diseases with significant unmet need

TEAM

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

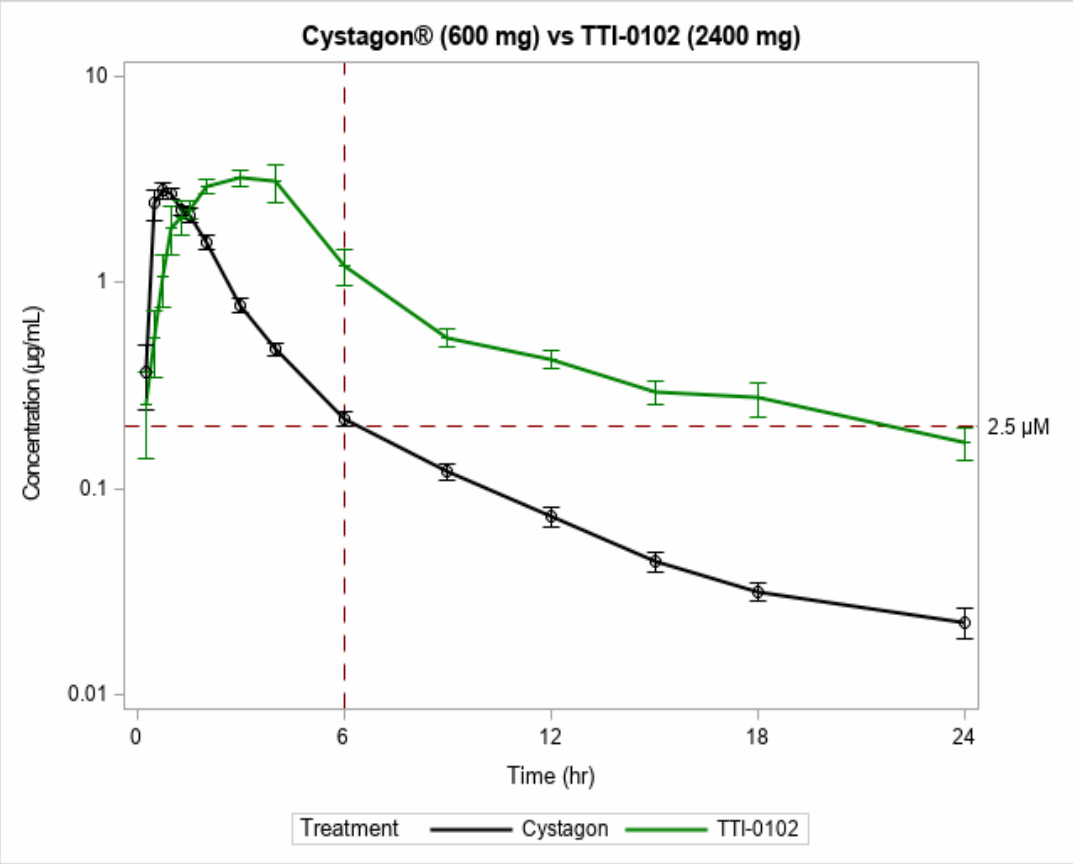
Deep experience at the intersection of rare disease & redox biology

- 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

PHASE 1 STUDY (PK/SAFETY)

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, Dr. Greenbaum (Emory)

THE CORE PROBLEM

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

Enteral Administration: For tube-dependent pediatric and adult patients, a soluble oral powder avoids constraints of a G-tube required in current delayed-release granule labeling.

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

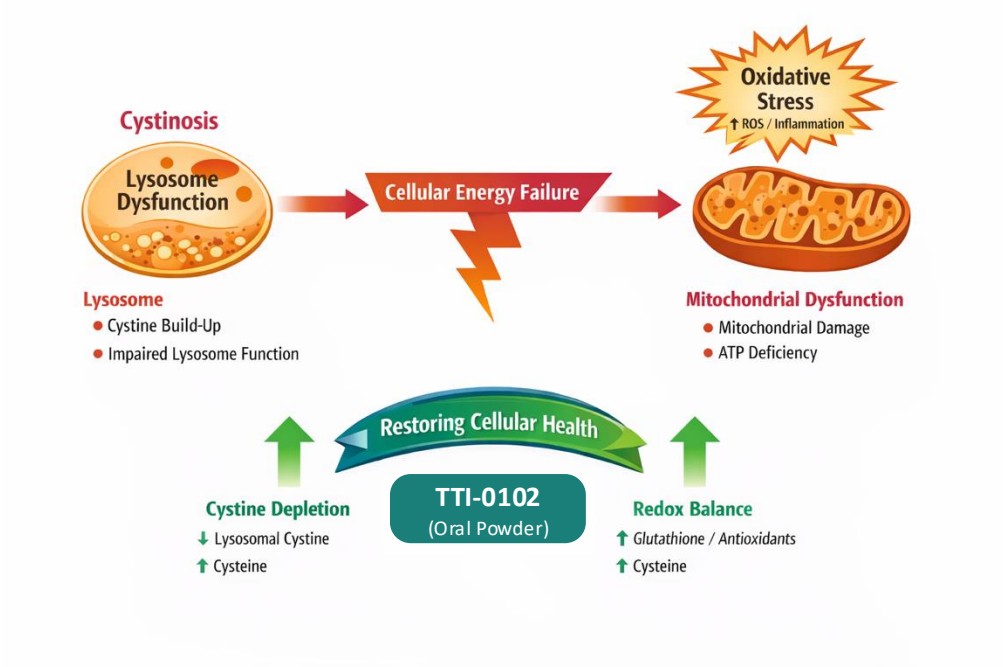
Severe pill fatigue; a rigid 5-hour daily fasting window; constant GI distress; severe social stigma from sulfurous breath and body odor.

A De-Risked Path to Cystinosis Phase 3

Rapid, Capital-Efficient Path to Registration

- Senior team that brought Procysbi® to market (Clinical, Regulatory, and CMC leadership)
- 505(b)(2) regulatory pathway established - leveraging 30 years of cysteamine safety and efficacy precedent
- Surrogate endpoint strategy: White blood cell (WBC) cystine suppression as the registrational surrogate biomarker (Langman et al., 2012 precedent)
- Investigator-initiated study with Dr. Greenbaum (Emory) to confirm 24-hour cystine control and inform pivotal protocol
- **Subsequent FDA meeting** to align on Phase 3 trial design and latitude under 505(b)(2)
- **Near-term catalysts:** Cystinosis study initiation in Q4 2026; data readout targeted in 1H 2027

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 and unmet need

Approximately **75,000 patients** affected in the U.S., with few approved therapies

Cysteine, glutathione and TTI-0102

- Low cysteine → reduced glutathione and oxidative stress
- TTI-0102 → lysosome → higher cysteine → glutathione (GSH) production

Platform potential

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

NEXT STEP IN VALIDATION

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

PLATFORM

Thiogenesis Development Pipeline

TTI-0102 Indication	Preclinical	Phase 1	Phase 2	Phase 3
Cystinosis (Lysosomal Storage Disease)	✓	✓	Investigator Initiated Study (Q4-2026)	tbd
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 Phase 3 trial in cystinosis
- Cystinosis positioned as the primary near-term value driver
- Leigh offers exposure to a substantially larger unmet mitochondrial disease opportunity
- Near-term catalysts capable of supporting a **meaningful valuation re-rating**

CORPORATE SNAPSHOT

Company Info

Shares Issued	69.9 million
Shares Fully Diluted	76.3 million
Insiders, f.d. (26%)	19.8 million
Share Price (09/25/2026)	$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