

A red bracket graphic consisting of two horizontal lines connected by vertical lines at the ends, framing the company name.

# THiogenesis THerapeutics

***Strong Orphan Platform + Rapid Clinical Path***

*TTI-0102: Next-generation cysteamine prodrug*

(TSXV: TTI / OTCQX: TTIPF)

December - 2025

# Forward Looking Statement

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This document and any attachments are intended for information purposes only and should not be construed as an offer or solicitation for the sale of securities. Statements in this presentation include forward-looking statements within the meaning of certain securities laws. These forward-looking statements include, among others, statements with respect to our objectives, goals and strategies to achieve those objectives and goals, as well as statements with respect to our beliefs, plans, objectives, expectations, anticipations, estimates and intentions. The words “expected to” “illustrate” “has the potential to” “will be”, “evaluating” “plans” “can be” “planning” “to predict” “potential” “may” “should” and words and expressions of similar import, are intended to identify forward-looking statements.

Results in early-stage clinical trials may not be indicative of full results or results from later stage or larger scale clinical trials and do not ensure regulatory approval. You should not put undue reliance on these statement or the scientific data presented as a number of important factors, many of which are beyond our control, could cause our actual results to differ materially from the beliefs, plans, objectives, expectations, anticipations, estimates and intentions expressed in such forward-looking statements. We do not undertake to update any forward-looking statements, whether written or oral, that may be made from time to time by us or on our behalf; such statements speak only as of the date made. The forward-looking statements included herein are expressly qualified in their entirety by this cautionary language.

# Thiogenesis - Summary

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**Clinical-stage biotech developing novel thiol-based prodrugs for high-need pediatric and orphan diseases**

- **Lead Compound, TTI-0102**, improved cysteamine prodrug that **targets mitochondrial dysfunction** *by increasing intracellular cysteine → GSH & taurine*
- **Leveraging decades of human data with** cysteamine across multiple diseases, de-risked mechanisms of action and well understood biology
- Active clinical programs in **MELAS, Leigh syndrome and pediatric MASH**, plus planning for Phase 3 in **cystinosis**
- Multiple near-term catalysts across 2026 and into 2027
- **Compelling Valuation:** < C\$40m with multiple value-creating catalysts

# Thiogenesis - Investment Highlights

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- **Clinically validated biology:** cysteamine-based drugs used in thousands of patients; strong GSH/antioxidant rationale
- **505(b)(2)** enables accelerated development and cost across all indications
- **Next generation cysteamine prodrug:** with improved GI tolerability + controlled sustained release, improves adherence and potential for once-daily dosing
- **Three de-risked indications:** with clear regulatory pathways (MELAS, Leigh syndrome, & Cystinosis) plus pediatric MASH expansion
- **Efficient capital plan:** multiple milestones achievable with modest spend
- **Leadership with multiple prior orphan-drug approvals** (*including Procysbi® - delayed release cysteamine*)

# Experienced Leadership

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*Leadership with deep experience in thiol pharmacology, rare pediatric diseases and orphan approvals*

## **Patrice Rioux, MD, PhD**

*Founder, CEO, Director*

- Leading authority in mitochondrial metabolism
- Former CMO / Head of Regulatory at Raptor; *led approval of Procysbi®*
- Clinical development leadership across rare and metabolic disorders

## **Christopher M. Starr, PhD**

*Chairman of the Board*

- Co-founder, BioMarin; *co-founder & CEO of Raptor (acquired for \$800M)*, Co-founder and Executive Chairman, Monopar
- 30+ years of experience building transformative orphan drug companies

BIOMARIN



Biogen

RepliGen

raptor  
pharmaceutical corp.

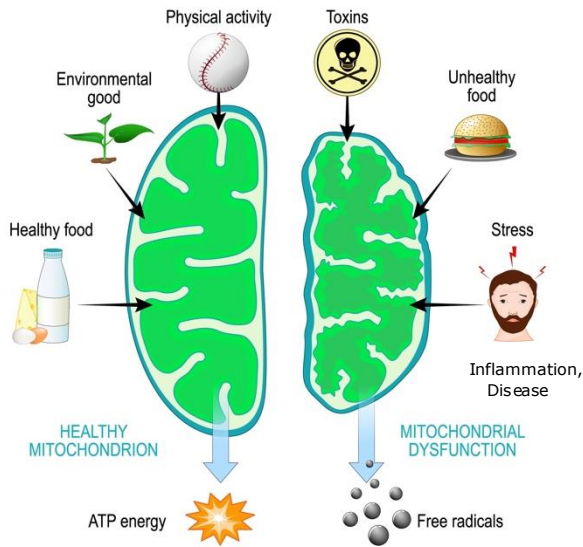
Naglazyme

ALDURAZYME<sup>®</sup>  
(LARONIDASE)

KUVAN<sup>®</sup>  
(sapropterin dihydrochloride)  
Tablets or Powder for Oral Solution

PROCYSBI<sup>®</sup>  
(cysteamine bitartrate)  
delayed-release capsules  
delayed-release oral granules

# TTI-0102 - Lead Compound

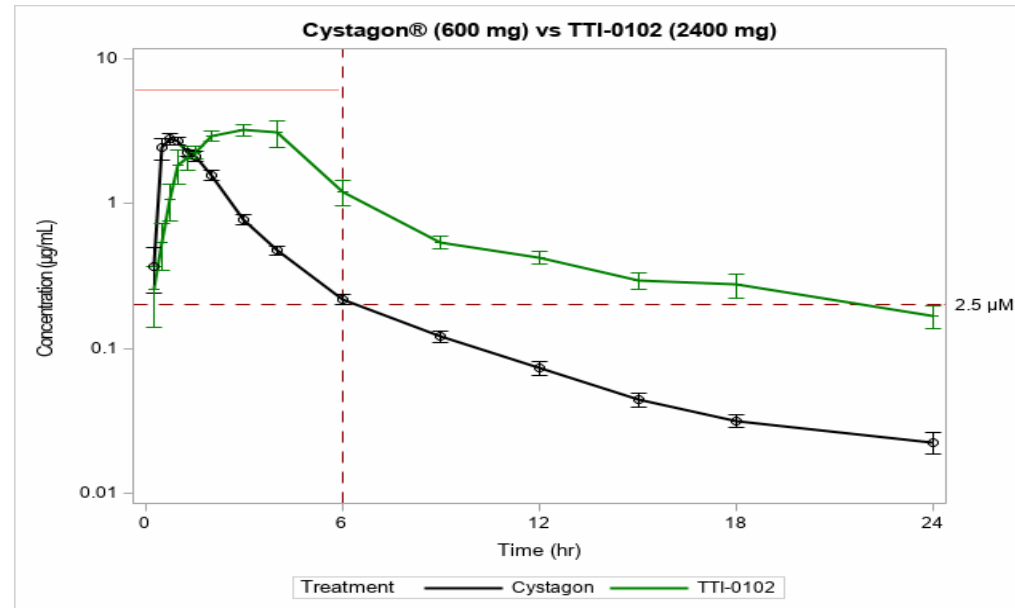


- **Asymmetric disulfide prodrug** that produces 2 cysteamine molecules + pantothenic acid (B5)
- **Controlled metabolic activation** → reduces peak-related GI related side effects common with existing cysteamine-based compounds
- **Extended PK** provides potential for *once-daily dosing, improving adherence and real-world use in chronic pediatric diseases*
- **Boosts intracellular cysteine**, enabling increased GSH (master antioxidant) and taurine to support mitochondrial function & neuronal stability
- **Applicable across multiple indications** where *oxidative stress, cysteine deficiency, and mitochondrial dysfunction* are key drivers

# TTI-0102 (Phase 1)

*A controlled-release next-generation cysteamine prodrug*

## Phase 1 Study (PK/Safety)



**Well-tolerated at 4x** therapeutic dosing level of generic cysteamine in Phase 1 (healthy adult volunteers)

# MELAS

**(Mitochondrial Encephalomyopathy, Lactic Acidosis & Stroke Like Episodes)**

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## ***MELAS: Strong biological rationale + Phase 2 underway***

- Rare inherited mitochondrial disease (m.3243A>G most common), diagnosed in children, with an estimated prevalence of 4.1:100,000
- Driven by oxidative stress, GSH deficiency and impaired energy metabolism
- *TTI-0102 increases GSH & taurine → further supporting neuronal stability*
- Phase 2 EU trial (NL + FR): randomized, placebo-controlled
- **Interim data:** demonstrated biological proof of concept, biomarker improvements, and mitochondrial antioxidant activity
- **Final 6-month data expected January 2026**



# Leigh Syndrome Spectrum (LSS)

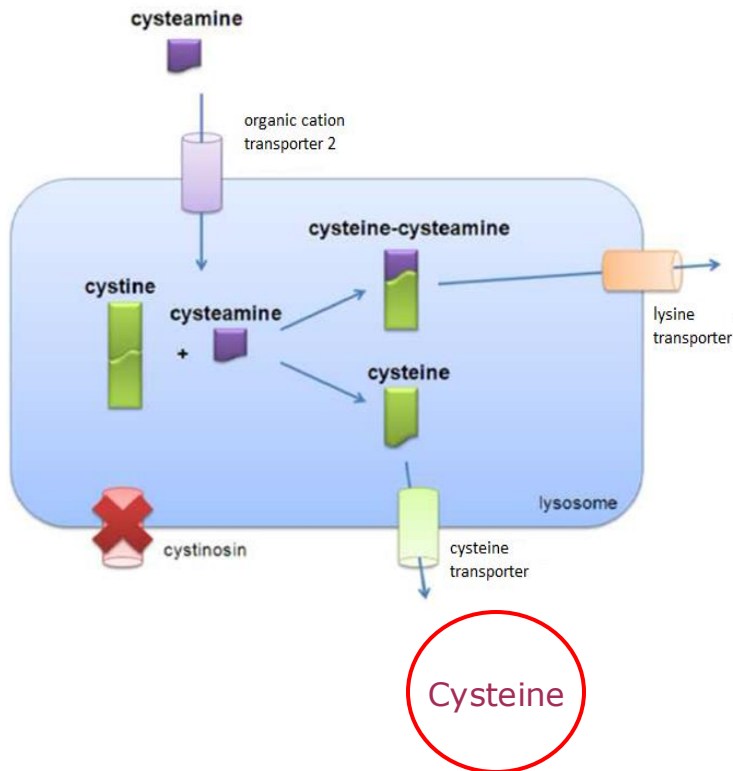
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*One of the most severe pediatric mitochondrial diseases*

- Severe pediatric mitochondrial disease; rapid neurodegeneration
- *TTI-0102 boosts GSH & taurine, reduces oxidative stress & supports neuronal function*
- **FDA-cleared IND (June 2025)** – in collaboration with leading US pediatric hospital
- ***Two-stage Phase 2a:***
  - Stage 1: randomized, placebo-controlled (9 pts)
  - Stage 2: pediatric open-label extension (6 pts)
- Dosing optimized based on MELAS interim results
- ***Initiation of Phase 2a trial anticipated in Q1-2026***

# Nephropathic Cystinosis

*Cystinosis: Established biology with clear Phase 3 path*



## Disease Biology

- Defective lysosomal transporter → toxic cystine crystal accumulation & progressive organ damage (i.e. kidneys)
- Cysteamine is clinically proven but limited by **GI intolerance and strict multi-dose schedules**

## TTI-0102 Rationale

- Designed for **improved tolerability & once-daily dosing potential**

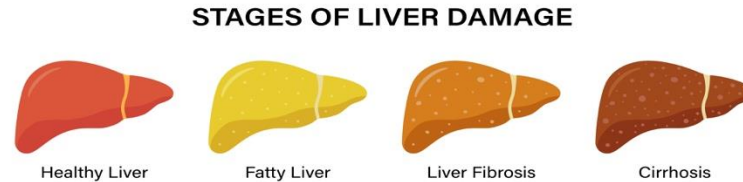
## Commercial Opportunity

- >\$300M addressable market
- ***Near-term Phase 3: IND planned for early 2026***

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

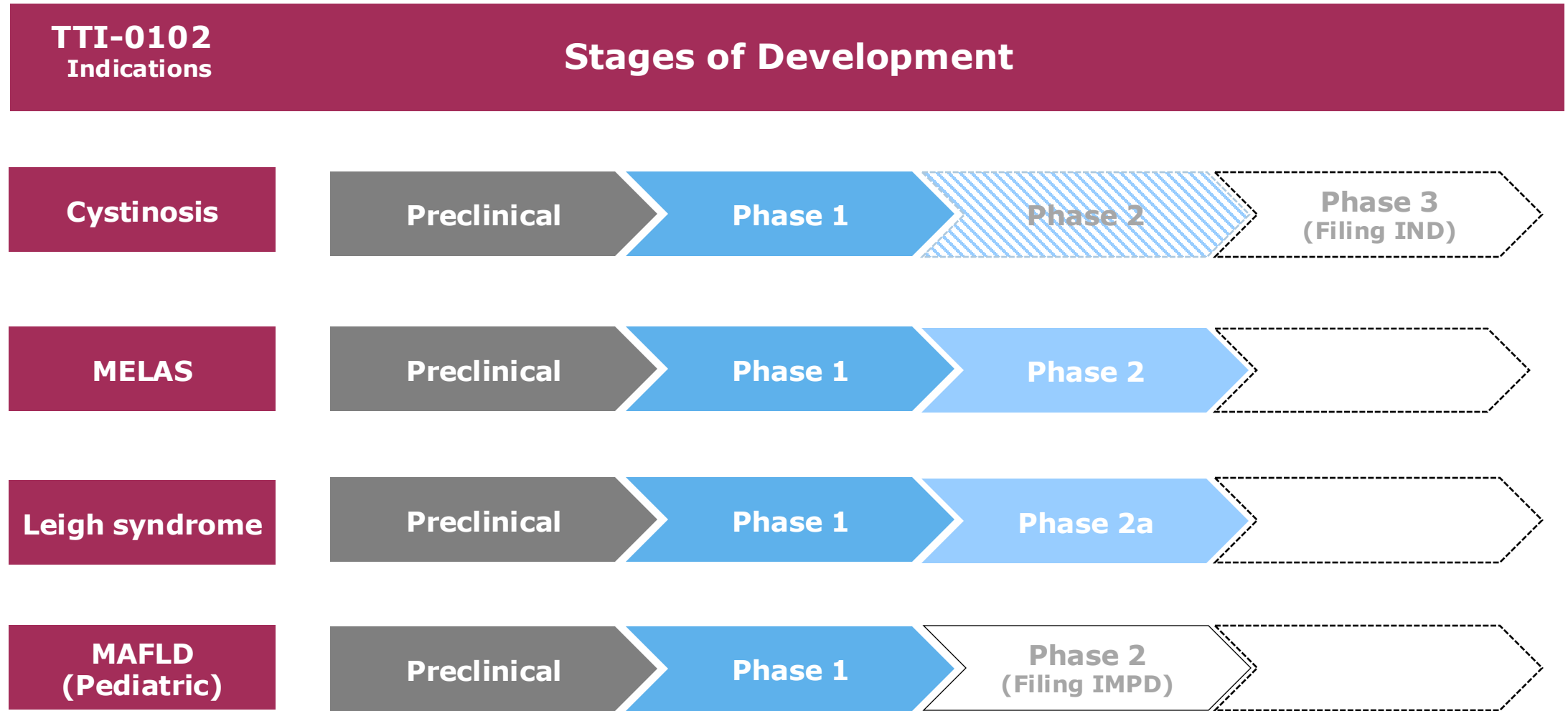
# Pediatric-Metabolic Dysfunction-Associated Steatohepatitis (MASH)

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- ~2-3% prevalence; driven by obesity, oxidative stress and mitochondrial dysfunction
- **Primary mechanism:** reduces mitochondrial oxidative stress and helps metabolize liver fat
- **Secondary mechanisms (fibrosis):**
  - **Cystine depletion** may down-regulate hepatic stellate cell activation
  - **Pantothenic acid (B5)** supports CoA pathways to metabolize fatty acids & aid tissue repair
- **NIH-CyNCh Trial** (DR-cysteamine) showed promising fat lowering signals but limited by dosing
- *TTI-0102 overcomes prior dosing limitations*
- ***IMPD submission planned for 2025 to initiate a Phase 2 (EU)***

# Thiogenesis Pipeline



# Thiogenesis – Upcoming Milestones

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## Potential milestones (6 months):

<b><i>MELAS</i></b>	➡	Final Phase 2 data – Jan 2026
<b><i>Leigh syndrome</i></b>	➡	Phase 2a patient enrollment Q1-26/data Q3-26
<b><i>Cystinosis</i></b>	➡	Phase 3 IND clearance Q1-26
<b><i>Pediatric MASH</i></b>	➡	Phase 2 IMPD cleared Q1-26

# Company Info

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## Thiogenesis Therapeutics

(TSXV: TTI / OTCQX: TTIPF)

**Shares Issued**

51.8 million

**Shares Fully Diluted**

56.9 million

**Insiders (32%)**

16.7 million

**Share Price (11/11/2025)**

\$0.72

**52 week high/low**

\$0.88/C\$0.51

**Market Cap.**

\$37.3 million

**Cash (09/30/2025)**

\$3.3 million

**Contact**

[info@thiogenesis.com](mailto:info@thiogenesis.com)

- Currency in Canadian dollars

# Companies of Interest

Name	Symbol	Disease	Stage	Market Cap	Notes
<b>Thiogenesis</b>	<b>TTI</b>	<b>MELAS</b>	<b>Ph.2</b>	<b>C\$34 mn</b>	<b>Anti-ox, Anti-Inflam.</b>
<i>Spruce</i>	SPRB	MPS IIIb	Ph. 2	US\$107 mn	Enz. Replacement
<i>Sagimet</i>	SGMT	Obesity/NASH	Ph 2	US\$224 mn	FASN
<i>Larimar</i>	LRMR	F. Ataxia	Ph. 2	US\$302 mn	Protein Replacement
<i>Zevra</i>	ZVRA	NP-C	NDA	US\$475mn	Enz. Signaling
<i>Monopar</i>	MNPR	Wilson's	NDA	US\$575 mn	Copper chelation
<i>Reata</i>	RETA	F. Ataxia	Approved	US\$7.0 bn	Anti-ox & Anti-Inflam.