



ØU Investor Konference

April 23, 2025

Emcitate[®] EMA approved and launch in Q2 2025, NDA submission planned for 2025

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Egetis: building an orphan drug commercial stage company



- 1 Focus on orphan diseases with proprietary asset **Emcitate®** (*tiratricol*) for the treatment of MCT8 deficiency, a detrimental condition with significant unmet medical need
- 2 Approved in EU in February 2025
Confirmation trial under way to complete US NDA dossier - planned submission 2025
- 3 The first and only approved drug for the treatment of MCT8 deficiency
A significant market opportunity & potential for expansion into RTH-beta
- 4 Preparing launch through focused in-house commercial organization in the EU and North America with partnership for RoW
- 5 A strong team with late-stage orphan clinical development, registration and commercialization experience

A strong regulatory status

ODD

Orphan drug designation - FDA, EMA
Market exclusivity 10y (EU) & 7y (US)

Fast
track

Fast track designation - FDA

PRV

Rare pediatric disease designation - FDA
Priority Review Voucher upon approval
Worth approx. \$150m

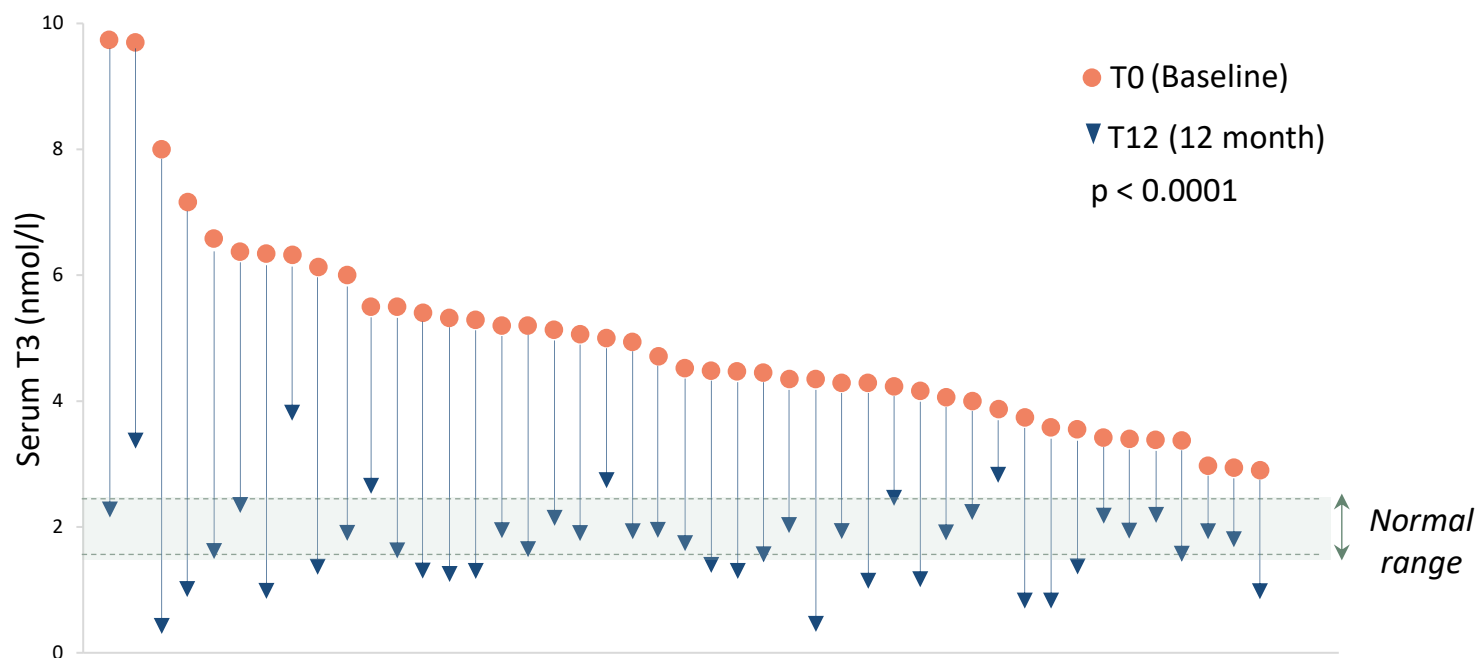
Listed on NASDAQ Stockholm (EGTX)
HQ in Stockholm, Sweden
~40 FTEs



Emcitate® treatment in patients with MCT8 deficiency is associated with survival benefits



In the Triac Trial I, *Emcitate* reached target level serum T3 & improvements in clinically relevant outcome measures



Emcitate is associated with a 3x lower risk of mortality in patients with MCT8 deficiency

Retrospective real-world cohort study in 228 patients - Abstract Aug. 2024

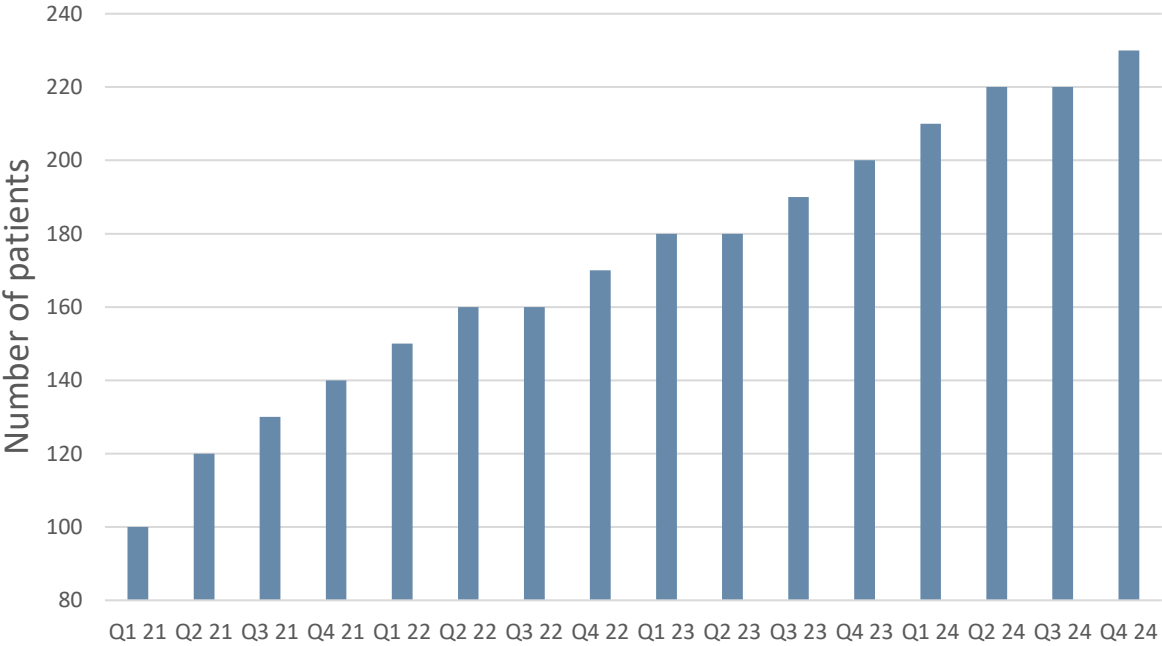
Key demonstrated clinical results

- ✓ Significant and durable reduction of T3 levels within the normal range
- ✓ Normalization of thyrotoxicosis in patients of all ages
- ✓ Improvement of bodyweight and cardiovascular status
- ✓ Beneficial effects are maintained or continue to improve over time, up to six years
- ✓ Benign safety profile

Emcitate already supplied globally in managed access programs

Successful traction through Managed access programs confirms the significant commercial potential for *Emcitate*

Patients Receiving *Emcitate* in Managed Access Programs



- Managed Access Programs allow early access to a medicine prior to regulatory marketing approval, in diseases with high unmet medical needs and no available treatment
- *Emcitate* is being supplied to around 230 patients in over 25 countries including US, EU

MCT8 deficiency key features

Estimated incidence:	1 case per 70k male
Median onset of symptoms:	4 months
Median age of diagnosis:	10 months
Patients surviving into adulthood:	70%
Severe intellectual disability:	100%
Ability to sit independently:	8%
Hypotonia, hypertonia	
& persistence of primitive reflexes:	90%
Severe underweight:	75%
Cardiac arrhythmias (PAC):	76%
Median life expectancy:	35 years
Patients dying in childhood:	~30%
Main cause of mortality:	Sudden cardiac death
Life long 24-hour care:	100%

Emcitate regulatory pathway in EU & US

Robust data set in an ultra rare genetic disease



Triac Trial I	EMC cohort study	Natural history	Triac Trial II	Survival study	ReTRIACt Trial
N=46	N=67	N=151	N=22	N~228	N=16
<ul style="list-style-type: none">Completed 2018 (Groeneweg, 2019)Open-label, international, multi-centre study	<ul style="list-style-type: none">Completed 2021 (van Geest, 2022)N= 27 from Triac Trial I & N= 40 new pts from managed access program	<ul style="list-style-type: none">Retrospective data, 2003 to 2019 (Groeneweg, 2020)	<ul style="list-style-type: none">Open-label, international, multi-centre studyFocus on neurocognition, but did not meet its primary endpoints96 weeks safety data in young patients	<ul style="list-style-type: none">Retrospective cohort dataComparing treated vs untreated patients on survivalAbstract at ETA by EMC	<ul style="list-style-type: none">N= 16Placebo controlledOngoing

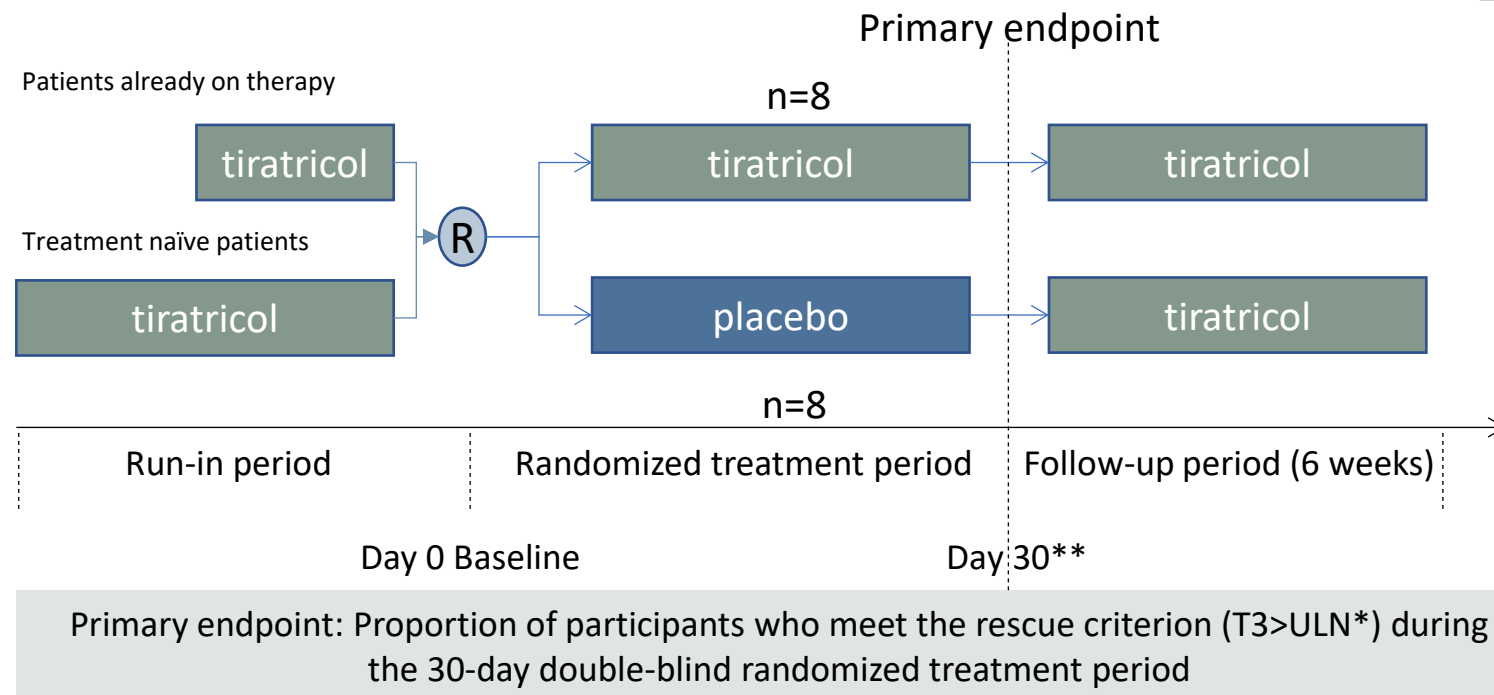
Data included in EMA MAA – EC approval Feb 2025

Data to be included in FDA NDA

Design of the ReTRIACt clinical trial

Requested by the FDA

- A 30-day, randomized placebo-controlled withdrawal study in at least 16 patients
- Design agreed with FDA
- The study allows for inclusion of patients that are already on therapy and patients that are treatment naïve
- Treatment naïve patients require a longer run-in period to stabilize T3 levels around normal range before randomization
- A higher proportion of treatment naïve patients will lead to an extended study duration



- 19 patients included so far, of which 11 patients have completed the randomized phase and 3 patients are in the run-in period. Several additional patients are currently being evaluated for screening
- 7 sites currently open, including new sites in Georgia, North Carolina, Texas and Florida.
- Recruitment will continue until at least 16 patients have completed the randomized phase

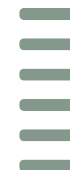
Egetis will update the market as soon as recruitment has been completed, and subsequently when top-line results.

NDA filing planned for 2025

* ULN: Upper Limit of Normal

** Randomized treatment period ends after 30 days or when rescue criterion (T3 > ULN) is met, whichever comes first

A phased EU launch through in-house commercial organization starting in Germany Q2



Wave 1

Initiated right after EU approval
Germany, France, Spain, Italy



Wave 2

Phased on a country-by-country approach
Rest of Europe



2025

2026

Pricing &
Reimbursement
processes

European Thyroid Association (ETA) guidelines published mid 2024, recommending *Emcitate* as long-term therapy for all patients with MCT8 deficiency

Building strong Expert base to advance management of MCT8 deficiency

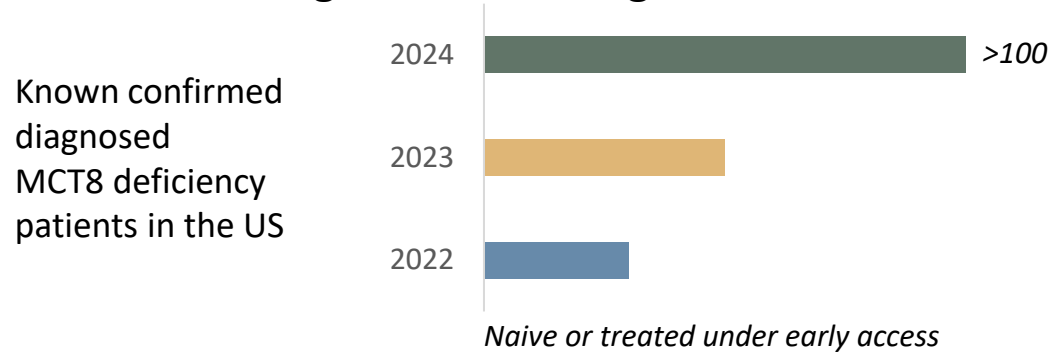
- Caregivers connected through international & national advocacy groups
- International KOLs & physicians at selected specialist centers
- Global strategy and local interactions with payers

Seizing opportunity for cost-effective value creation

- High disease burden of MCT8 deficiency also confirmed by Egetis sponsored Caregiver and vignette studies
- Targeted stakeholder interactions
- Efficiency gains through global-country team coordination

US commercial preparedness activities under way

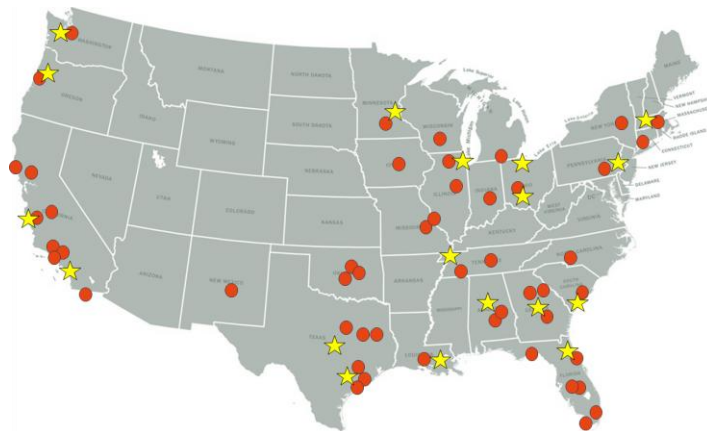
Accelerate patient finding efforts by integrating advanced data-driven insights into existing initiatives



Cover major US centers through pre-commercial initiatives

● Hospitals with known MCT8 deficiency patients

★ *Emcitate* Expanded Access Program sites



Expanded Access Program, requested by FDA, is a significant asset to both patients and Egetis launch readiness

- Providing early and sustainable access to therapy
- Exposing physicians to *Emcitate* prior to commercial approval
- Collecting real world data to support payer and regulatory communications

Patient-centric implementation

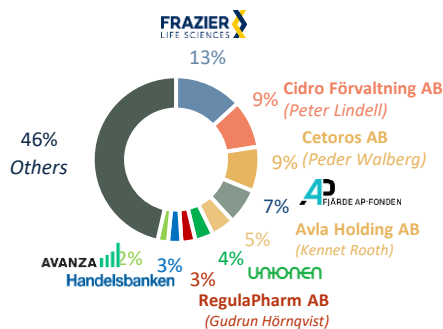
- Partnership with AnovoRx
- Personalized support; drug delivered directly to patient home

Strong financial foundation for strategic execution

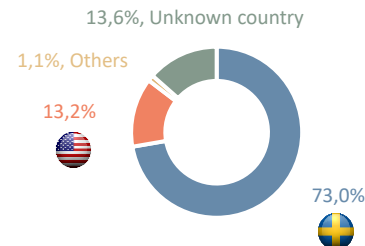
Solid cash position

- **Cash position December 31, 2024:** SEK 351 million
- **Number of outstanding shares:** 359,238,126
- **Market Cap:** ~SEK 1.6 billion*
- **Listing venue:** Nasdaq Stockholm, Main Market
- **Ticker:** EGTX

% capital by shareholders



% capital by geographical distribution



Share issuance in Sep. 2024 for SEK 300m (USD 30m)

- Led by Frazier Life Sciences (USD 10 million)
- Supported by international and Swedish healthcare funds
- Subscription at market price

- Clinical development of *Emcitate* for US market authorisation
- Preparatory *Emcitate* launch activities in Europe
- Continued build-up of commercial and medical affairs infrastructure for *Emcitate* commercialization in the EU and US

Note: * March 4, 2025

Upcoming value enhancing key milestones 2025-2026



Emcite®

2025-2026

MCT8
deficiency

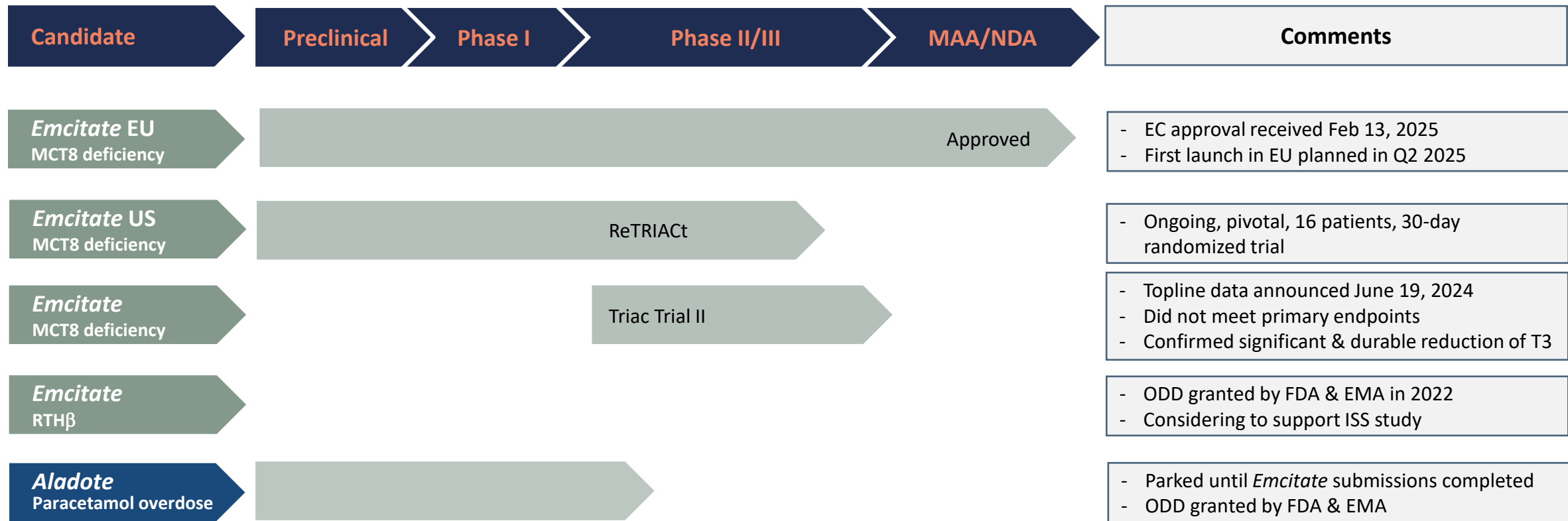
- EU launch, in the first country, Germany, during the second quarter of 2025
- Topline results ReTRIACt for US NDA
- Filing US NDA 2025 – fast track designation
- Middle East & North Africa partnership/s
- Japan – Development plan agreed with PMDA
- Potential grant of US Patent - Processes and compounds
- US approval and launch
- US Rare Pediatric Disease Priority Review Voucher

RTH-beta

- Potential initiation of Investigator Initiated Study - Egetis Industry collaborator

Pipeline overview

Emcitate – European Commission approval Feb 13, 2025



Thank you!

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