



Corporate presentation

November 2024

An integrated orphan drug company, focusing on late-stage development for commercialization

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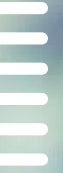
Agenda



1. An integrated orphan drug company, focusing on late-stage development for commercialization
2. *Emcitate*
 - Overview of MCT8-deficiency
 - Clinical experience with *Emcitate*
 - Regulatory pathway to submissions in EU and US
 - Commercial opportunity
3. *Aladote**
 - Paracetamol/Acetaminophen overdose and clinical experience with *Aladote*
 - Regulatory pathway to submissions in EU and US
 - Commercial opportunity
4. The orphan drug segment
5. Summary
- A. Appendix

*In-house development of *Aladote* parked until *Emcitate* NDA/MAA submissions have been completed

WE CARE
FOR THE RARE



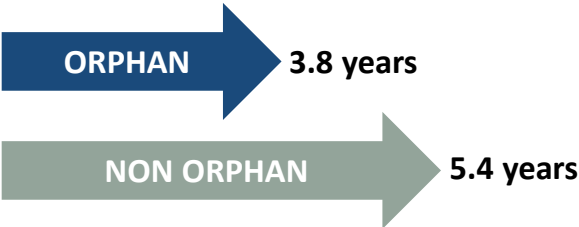
1.

An integrated orphan drug company, focusing on late-stage development for commercialization

Orphan drug segment – a highly attractive opportunity

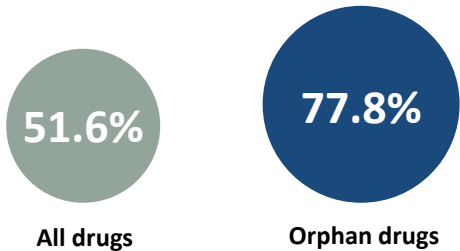
Shorter clinical development time¹

Phase II to launch Average # of years



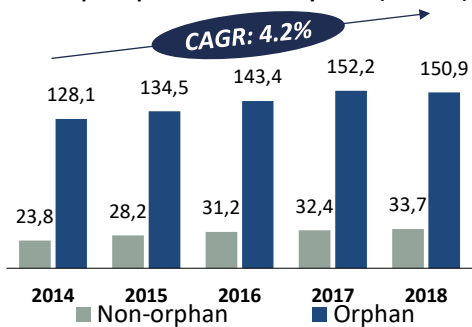
Higher probability of success³

Phase III to approval
POS in metabolic/endocrinology indications



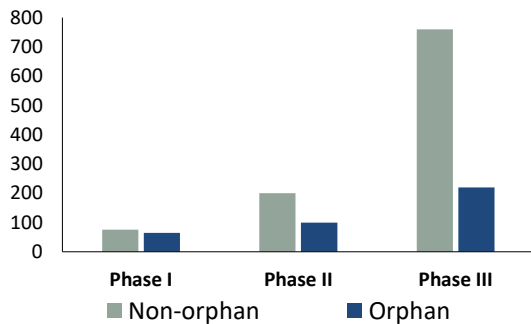
Higher attainable prices²

Mean cost per patient and year (USDk)

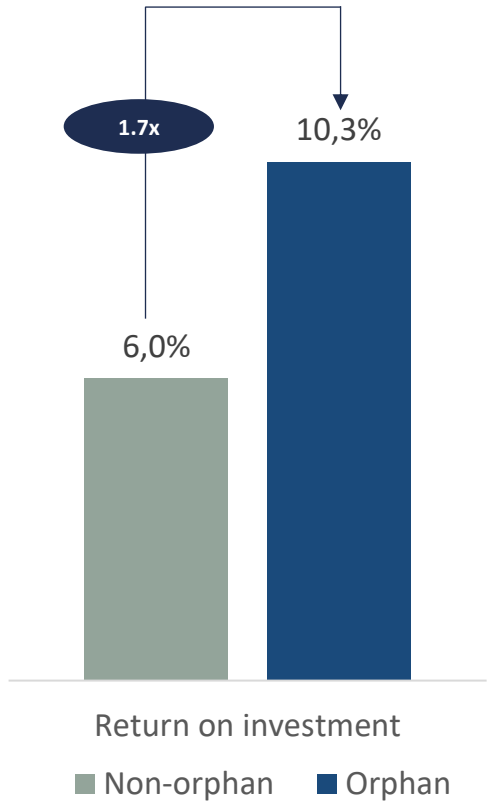


Fewer patients for clinical trials⁴

Patients per trial



Orphan drugs attractive returns⁵



Source: (1) Orphan drug development: an economically viable strategy for biopharma R&D, Meekings, Williams & Arrowsmith, 2012; (2) EvaluatePharma; (3) Estimation of clinical trial success rates and related parameters, C. Wong, K. Siah, A. Lo, Biostatistics, 2019; (4) BioMed Central; (5) EvaluatePharma Orphan Drug Report 2013

Note: Orphan Drugs: Populations of less than 5/10,000 inhabitants in the EU or <200,000 inhabitants in the US

An integrated orphan drug company, focusing on late-stage development for commercialization



1

Dedicated orphan drug company
Two late-stage assets: ***Emcitate*** and ***Aladote****

2

Emcitate MAA filed in **October 2023**
Pivotal trial for ***Emcitate*** NDA is ongoing

3

Highly attractive **orphan drug segment**

4

Plan to **launch** through **small in-house commercial** organization in the EU and North America

5

Strong team with late-stage orphan clinical development, registration and commercialization experience from:



Listed on NASDAQ Stockholm (EGTX)

HQ in Stockholm, Sweden

~40 FTEs

*In-house development of *Aladote* parked until *Emcitate* submissions have been completed

Building a sustainable orphan drug company

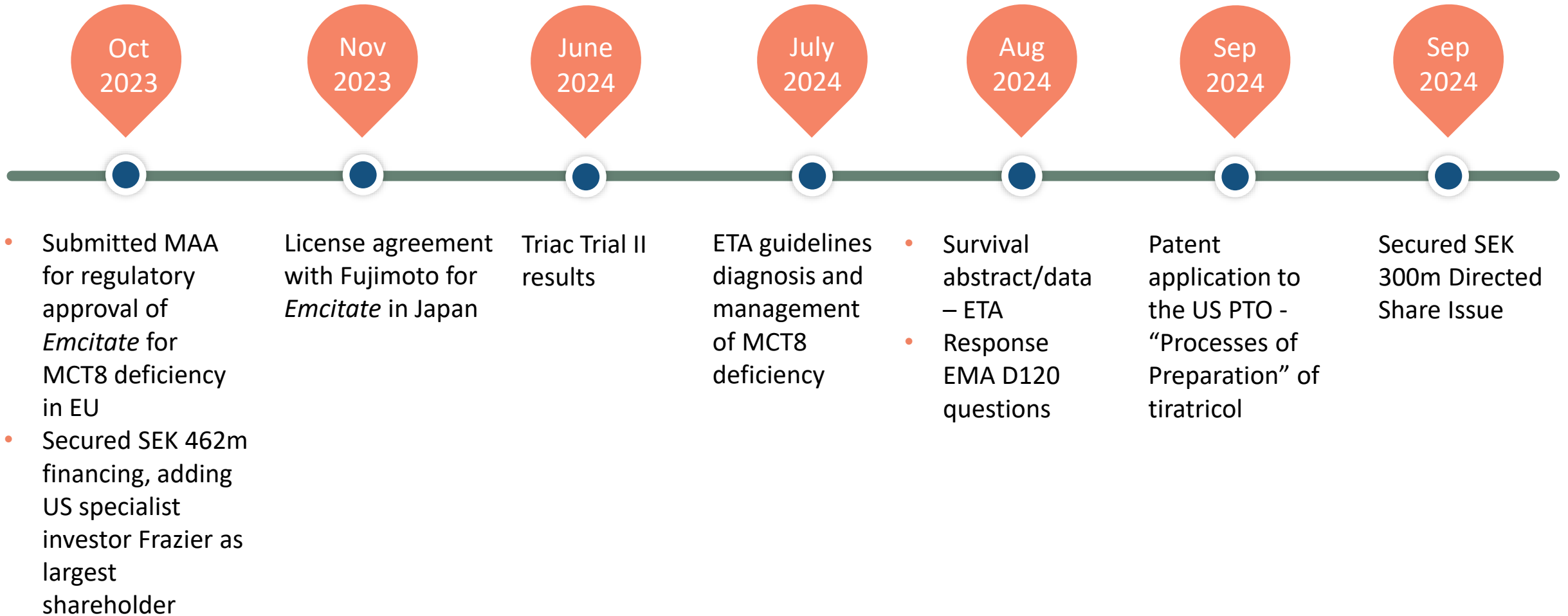
- Successfully develop *Emcitate* for EU & US approvals in 2024/25 and *Aladote* post 2026
- Commercialize *Emcitate* and *Aladote* through an inhouse organization in Europe/ North America and partnerships in RoW
- Realize the full potential of our products via life-cycle management
- Ensure fast and broad access to our products for the benefit of patients worldwide
- Identify further assets that address the significant unmet medical need for patients with rare diseases
- Provide an open culture that encourages Collaboration, Courage & Commitment
- Egetis financial objective is to create increased value for shareholders in the long term

To bring unique therapies to patients with rare diseases that improve and extend life

To create value for patients, society and shareholders by developing and providing a portfolio of unique products for the treatment of rare diseases with substantial medical need



Several important milestones over the last 12 months



Submission of application for regulatory approval in EU (MAA) for *Emcitate* for treatment of MCT8 deficiency

- **Marketing Authorisation Application (MAA) submitted and validated by the EMA in October**
- Application validated and found to be complete by the EMA on October 27, starting the clock for formal regulatory review
- Response to D180 List of Outstanding Issues submitted
- The median review time for MAAs in the EU is around 13-14 months
- Orphan Drug Designation will provide 10 years of market exclusivity within the EU following approval of MAA



Egetis submits marketing authorisation application for *Emcitate* for treatment of MCT8 deficiency to the European Medicines Agency

October 9, 2023

- Submission based on efficacy data from two studies in a total of 86 MCT8 deficiency patients with up to 6 years of treatment with *Emcitate*
- To date an accumulated 500 patient years of *Emcitate* treatment have been gathered in patients with MCT8 deficiency
- If approved, *Emcitate* would become the first treatment for MCT8 deficiency

Stockholm, Sweden, October 9, 2023. Egetis Therapeutics AB (publ) ("**Egetis**" or the "**Company**") (Nasdaq Stockholm: EGTIX), today announced that the Company has submitted a marketing authorisation application (MAA) to the European Medicines Agency (EMA) for *Emcitate* (tiratricol) for the treatment of MCT8 deficiency. This is an important step towards bringing the first approved treatment for MCT8 deficiency to patients and a transformative milestone for the Company.



Egetis announces EMA validation of Marketing Authorisation Application for *Emcitate* for the treatment of MCT8 deficiency

October 27, 2023

Stockholm, Sweden, October 27, 2023. Egetis Therapeutics AB (publ) ("**Egetis**" or the "**Company**") (Nasdaq Stockholm: EGTIX), today announced that its Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for *Emcitate* (tiratricol) for the treatment of MCT8 deficiency has been validated. On October 9, 2023, Egetis announced the submission of the MAA. EMA performs a validation of the applications it receives. The objective is to make sure all essential regulatory elements required for scientific assessment are included in the application prior to the start of the review procedure. The *Emcitate* MAA is as of October 26, 2023, under review by the Committee for Medicinal Products for Human Use (CHMP).

Egetis secured long-term financing of SEK 462m and added top-tier US specialist investor as largest shareholder

Announcement published on October 10, 2023



- **Unique combined long-term financing, comprising SEK 172m private placement at a premium and SEK 290m debt financing**
 - First in its class in a Swedish biotech setting, limiting dilution to existing shareholders and strengthening shareholder base



- **Private placement led by top-tier US healthcare specialist investor Frazier Life Sciences**
 - Demand for the new shares significantly exceeding the size of the private placement
 - Frazier Life Sciences new largest strategic shareholder in EGTX and brings significant sector expertise



- **SEK 290m debt financing obtained from BlackRock (formerly Kreos)**
 - Divided into two tranches, EUR 10m (“Tranche A”) and EUR 15m (“Tranche B”) which will become available provided that the Company reaches certain milestones, inter alia related to the phase III ReTRIACt study for Emcitate for Tranche B.
 - Egetis drew down Tranche A of the Debt Financing on November 30, 2023

Advancing rest of world with license agreement with Fujimoto for Emcitate in Japan



- **Highly suitable partner in Fujimoto**
 - Private company in Osaka, Japan, founded in 1933
 - Significant experience from successfully registering and launching medicines for Blood, Neurological and Orphan diseases in Japan
- **Egetis retains significant share of future revenues in Japan**
 - Upfront, development & regulatory milestones of total JPY 600m (SEK 45m)
 - In addition, Fujimoto will finance the necessary development in Japan and be responsible for regulatory interactions
 - Egetis retains ~1/3 of future revenues



Egetis announces exclusive license agreement with Fujimoto to develop and commercialize Emcitate in Japan

November 10, 2023

Stockholm, Sweden, November 10, 2023. Egetis Therapeutics AB (publ) ("**Egetis**" or the "**Company**") (Nasdaq Stockholm: EGTJ), today announced that the Company, through its wholly-owned subsidiary Rare Thyroid Therapeutics International AB, has entered into an exclusive license agreement with Fujimoto Pharmaceutical Corporation ("**Fujimoto**") to develop and commercialize *Emcitate* (tiratricol), for the treatment of MCT8 deficiency, in Japan. Under the terms of the agreement Egetis grants Fujimoto exclusive development and commercialization rights to *Emcitate* for the treatment of MCT8 deficiency in Japan. Fujimoto will pay upfront, development, and regulatory milestones amounting to JPY 600 million (approximately SEK 45 million). Egetis will supply Fujimoto with product in semi-finished form and will receive approximately one third of the applicable income from Fujimoto. Fujimoto will also finance the development program needed for *Emcitate* in Japan, which will be clarified after discussions with the Pharmaceuticals and Medical Devices Agency (PMDA). As a future marketing authorisation holder (MAH) Fujimoto will be responsible for regulatory interactions with the PMDA.

European Thyroid Association (ETA) recommends tiratricol as long-term therapy for all patients with MCT8 deficiency



- ETA recommends the use of tiratricol as long-term therapy for all patients with MCT8 deficiency, and for certain patients with RTH-beta.
- Inaugural 2024 Guidelines were commissioned by the Executive Committee of the ETA and developed by an independent team of experts.



European Thyroid Association recommends tiratricol (Emcitate®) as long-term therapy for all patients with MCT8 deficiency in new guidelines

July 17, 2024

Stockholm, Sweden, July 17, 2024. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (Nasdaq Stockholm: EGTX), today announced that the European Thyroid Association (ETA) has published new guidelines recommending the use of tiratricol (TRIAC or Emcitate®) as long-term therapy for all patients with MCT8 deficiency, and for certain patients with Resistance to Thyroid Hormone (RTH)-beta, as further outlined in the guidelines.

There are currently no approved treatments for MCT8 deficiency or RTH-beta. Egetis has obtained orphan drug designation for tiratricol for the treatment of MCT8 deficiency and RTH-beta in the EU and the USA, and has submitted a marketing authorisation application in the EU, which is currently under review by the European Medicines Agency.

These inaugural 2024 *European Thyroid Association Guidelines on diagnosis and management of genetic disorders of thyroid hormone transport, metabolism and action* were commissioned by the Executive Committee of the ETA and developed by an independent team of experts. The guidelines can be accessed here:

<https://etj.bioscientifica.com/view/journals/etj/aop/etj-24-0125/etj-24-0125.xml>

New study reports tiratricol (Emcitate®) treatment in patients with MCT8 deficiency is associated with survival benefits



- Abstract published ahead of the ETA Annual Meeting reports that treatment with tiratricol (Emcitate®) in patients with MCT8 deficiency is associated with a 3x lower risk of mortality.
- Retrospective real-world cohort study investigated the effects of tiratricol on mortality in 228 patients with MCT8 deficiency.
- Tiratricol-treated patients had an approximately three times lower risk of all-cause mortality (Hazard Ratio= 0.28, 95% Confidence Interval= 0.09–0.91, p-value <0.05).



New data shows tiratricol (Emcitate®) treatment in patients with MCT8 deficiency is associated with survival benefits

August 21, 2024

- Abstract by F. van der Most et al. published ahead of the 46th Annual Meeting of the European Thyroid Association, to be held in Athens, Greece, on September 7-10, 2024.
- An international real-world cohort study included data from 228 patients collected from 173 sites in 48 countries.
- Treatment with the investigational drug tiratricol (Emcitate®) in pediatric and adult patients with MCT8 deficiency is associated with an approximately three times lower risk of mortality. This corroborates previous findings indicating that tiratricol sustainably alleviated key clinical features resulting from peripheral thyrotoxicosis.

Stockholm, Sweden, August 21, 2024. Egetis Therapeutics AB (publ) (“**Egetis**” or the “**Company**”) (Nasdaq Stockholm: EGTX), today announced the content of an abstract by Dr Floor van der Most and co-authors, Erasmus Medical Center, Rotterdam, The Netherlands, published ahead of the 46th Annual Meeting of the European Thyroid Association, to be held in Athens, Greece, on September 7-10, 2024. In the Abstract, treatment with the investigational drug tiratricol (Emcitate®) in paediatric and adult patients with MCT8 deficiency is associated with an approximately three times lower risk of mortality compared to MCT8 deficiency patients not treated with tiratricol.

Egetis submits patent application to the USPTO



- Patent application for “Processes of Preparation” of tiratricol
- Processes and compounds described in the patent application
- If granted, this would be a significant patent for Egetis



Egetis submits a patent application to the United States Patent and Trademark Office for “Processes of Preparation” of tiratricol

Stockholm, Sweden, September 19, 2024. Egetis Therapeutics AB (publ) (“Egetis” or the “Company”) (Nasdaq Stockholm: EGTX), today announced that it has submitted a patent application with the United States Patent and Trademark Office (USPTO) for “Processes of Preparation” of tiratricol. If granted, this would be a significant patent Egetis has obtained for the investigational drug tiratricol.

Tiratricol is an endogenously available metabolite of thyroid hormone, with similar bioactive properties as the active thyroid hormone T3. Tiratricol enters the cell independently of the monocarboxylate transporter 8 (MCT8), bypassing the pathophysiologic defect in MCT8 deficiency. Clinical trials for the use of tiratricol for the treatment of MCT8 deficiency are ongoing and in October 2023 Egetis submitted a marketing authorisation application (MAA) in the EU. Accordingly, new and more efficient synthetic routes leading to tiratricol are needed. The processes and compounds described in the patent application help meet these and other needs.

Egetis carried out directed share issuances amounting to SEK 300 million (USD 30 million)

Announcement published on September 30, 2024

- Led by Frazier Life Sciences with a USD 10 million investment.
- Supported by international and Swedish specialist healthcare funds.
- Subscription price at market price.



Egetis Therapeutics has successfully carried out directed share issuances amounting to SEK 300 million

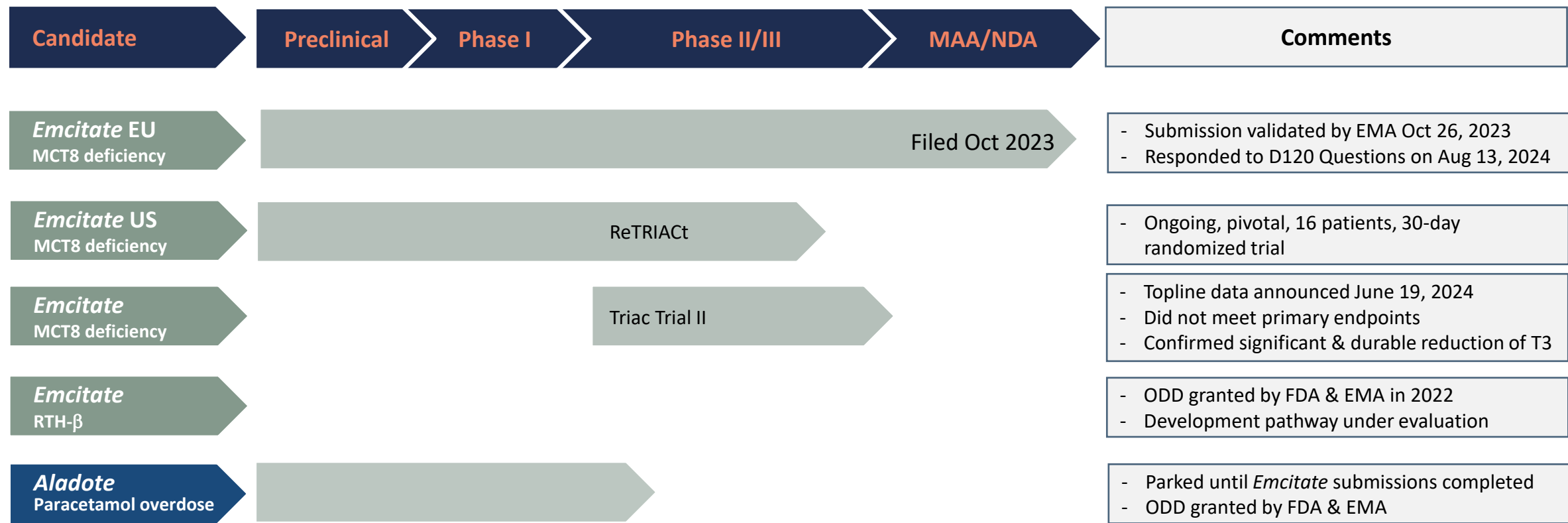
September 30, 2024

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Stockholm, Sweden, September 30, 2024. The Board of Directors of Egetis Therapeutics AB (publ) (“Egetis” or the “Company”) (Nasdaq Stockholm: EGTX) has resolved on directed share issuances of in total 66,666,667 new ordinary shares at a subscription price of SEK 4.50 per share, corresponding to a 0.1 percent premium to the 5 day volume weighted average price (VWAP) preceding this announcement (the “Directed Issue”), through which the Company receives SEK 300 million (approximately USD 30 million) before transaction costs. The Directed Issue was oversubscribed and included both existing and new international and Swedish institutional investors. It was led by US healthcare investor Frazier Life Sciences with a USD 10 million investment, and supported by the international healthcare specialist Invus (USA/France), Platinum Asset Management (Australia), The Fourth Swedish National Pension Fund, Handelsbanken Fonder AB through the investment fund Hälsovård Tema (Sweden), Unionen (Sweden), HealthInvest Partners AB (Sweden) and Cidro Förvaltning AB (Sweden).

Pipeline overview

Emcitate MAA filed in October 2023



Two highly promising orphan drug candidates

Emcitate® – Therapy for MCT8 deficiency

- MCT8 deficiency affects ~1:70,000 males: high unmet medical need, no available treatment. No competing sponsored products in clinical development
- Orphan Drug Designation in EU & US
- US Rare Pediatric Disease Designation, eligible for **Priority Review Voucher**. Fast track designation granted by FDA
- Triac Trial I (Phase IIb) completed with **significant** and **clinically** relevant effects on **T3 levels** and **chronic thyrotoxicosis**
- Real-world data published **2021 confirms long-term efficacy and safety** of *Emcitate*
- **MAA** filed in **October 2023**
- **Pivotal ReTRIACt trial for US NDA is ongoing**. A 30 days placebo-controlled trial in 16 patients to verify the results on T3
- **Triac Trial II**: Topline results announced June 19, 2024. Did not meet primary endpoints. Confirmed the significant and durable reduction of T3 levels in all patients
- Over **220 patients** are being **treated** with *Emcitate* in managed access programs— Expanded Access Program implemented in the US as requested by the FDA

Aladote® – To prevent acute liver injury caused by paracetamol poisoning*

- Paracetamol poisoning is one of the most common overdoses with >175,000 hospital admissions globally per annum
- No adequate treatment exists for increased risk patients
- Orphan drug designation (ODD) granted in the US & EU
- Successful results from Phase Ib/IIa study in paracetamol overdosed patients
- Pivotal Phase IIb/III study planned for marketing authorization application in both US and EU
- No competing products in clinical development
- In-house development parked until *Emcitate* submissions have been completed for MCT8 deficiency

*In-house development of *Aladote* parked until *Emcitate* submissions have been completed

2.

Overview of MCT8 deficiency

MCT8 deficiency results in dysfunctional thyroid hormone trafficking

MCT8 deficiency has two co-manifestations

New Research Sheds Light on Thyroid Hormone Transport

- In 2003, MCT8 was identified as one of the first thyroid hormone transporters
 - Previously, thyroid hormone was incorrectly believed to be able to passively cross cellular membranes, without the need for a specific transporter
- Several additional transporters have been identified with preferential distribution across different tissue types and cells

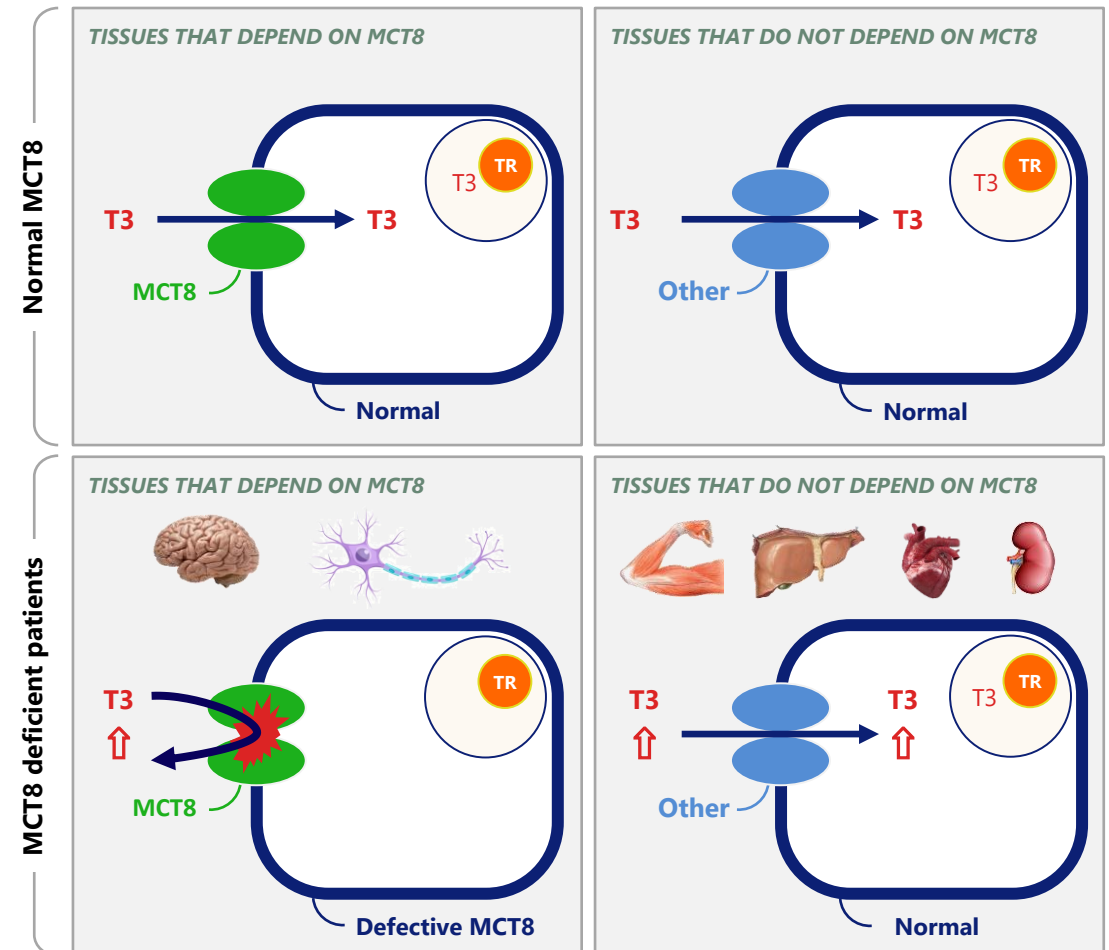
MCT8 Plays a Key Role in Neurocognitive Development

- MCT8 is the only thyroid hormone transporter in the cells of the blood brain barrier and neurons
 - The human brain is dependent on thyroid hormone for its normal development. Absence of thyroid hormone in the CNS leads to disruption of neurocognitive development and results in severe neurocognitive and motor impairment

And Causes Many Additional Symptoms

- Disrupted thyroid hormone homeostasis leads to an increase of peripheral serum T3 levels
- Tissues dependent on transport other than MCT8 suffer from too high levels of thyroid hormone:
 - Increased heart frequency, blood pressure and arrhythmias
 - Severe wasting and weight loss
 - Impaired liver / kidney function
 - Altered bone metabolism and blood lipids
 - Increased risk of sudden and premature death

MCT8 deficiency results in simultaneous too high and too low thyroid hormone levels – causing system wide issues



MCT8 deficiency: a detrimental condition with significant unmet medical need



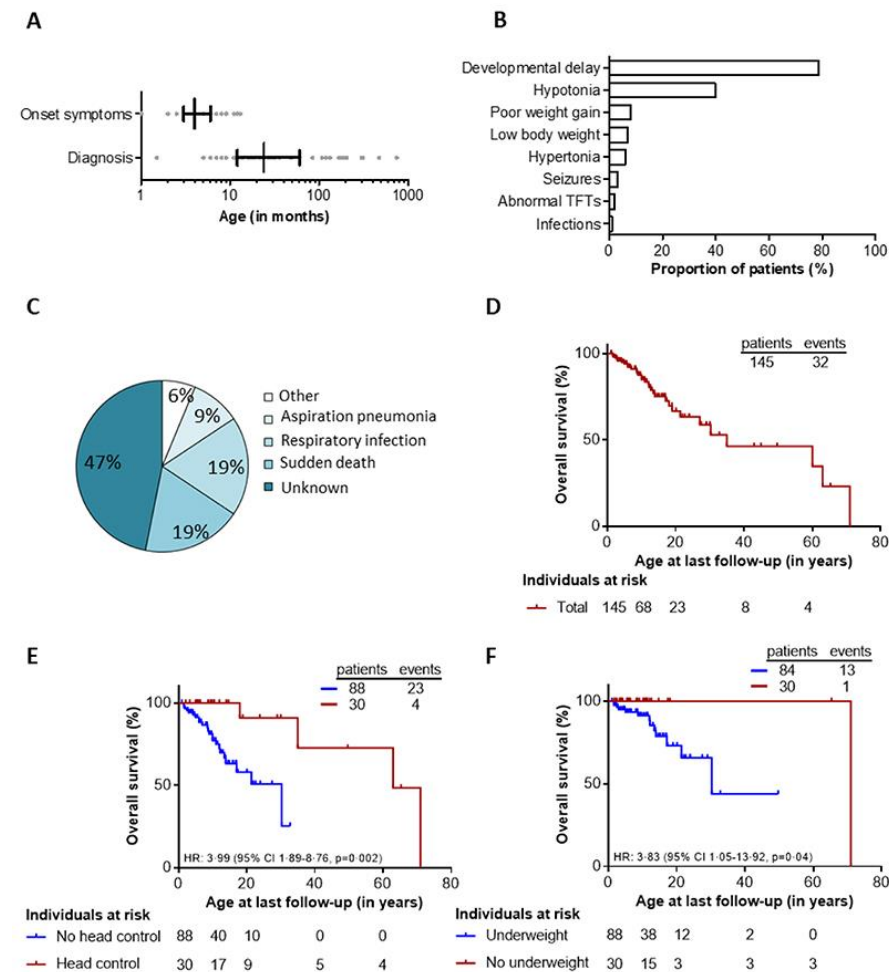
What is MCT8 deficiency?	What does it mean?	What are the challenges?	How do you manage the disease?	Quick facts from natural history ²																						
<ul style="list-style-type: none">Genetic X-linked disorderImpaired thyroid hormone trafficking across cellular membranesMCT8 is a key thyroid hormone transporter in the bodyPrevalence 1:70,000 males <div></div> <p>Patients with MCT8 Deficiency¹⁾</p>	<ul style="list-style-type: none">Non-functional MCT8 protein: T3 cannot cross blood-brain-barrierLow amounts of thyroid hormone in the brain & CNSDisrupted feedback loop results in a compensatory increase in circulating thyroid hormone <div></div> <ul style="list-style-type: none">Simultaneous too high & too low thyroid hormone in different tissues	<ul style="list-style-type: none">Patients appear normal at birthInitial symptoms within the first months of lifeSevere intellectual disabilityMost patients never able to sit or walk; limited ability to communicateLife-long morbidity: agitation, CV symptoms, wasting & impaired life expectancy <div></div> <ul style="list-style-type: none">Heavily dependent on caregivers resulting in very high disease burden	<ul style="list-style-type: none">No available therapyEasy diagnosis once considered with readily available, low-cost lab-testLarge proportion of patients remain undiagnosed with significant delay to diagnosis <div></div> <ul style="list-style-type: none">Significant unmet medical need: humanitarian, health economic, societal	<table><tr><td>Median onset of symptoms:</td><td>4 months</td></tr><tr><td>Median age of diagnosis:</td><td>10 months</td></tr><tr><td></td><td>(prior to 2017: 24 months)</td></tr><tr><td>Patients surviving into adulthood:</td><td>70%</td></tr><tr><td>Severe intellectual disability:</td><td>100%</td></tr><tr><td>Ability to sit independently:</td><td>8%</td></tr><tr><td>Hypotonia, hypertonia & persistence of primitive reflexes:</td><td>90%</td></tr><tr><td>Severe underweight:</td><td>75%</td></tr><tr><td>Cardiac arrhythmias (PAC):</td><td>76%</td></tr><tr><td>Median life expectancy:</td><td>35 years</td></tr><tr><td>Life long 24-hour care:</td><td>100%</td></tr></table>	Median onset of symptoms:	4 months	Median age of diagnosis:	10 months		(prior to 2017: 24 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	Life long 24-hour care:	100%
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Note: 1) Picture from Schwarz et al; Clin Endocrinol & Met 2007; 2) Groeneweg et al, Lancet Diabetes & Endocrinology, 2020

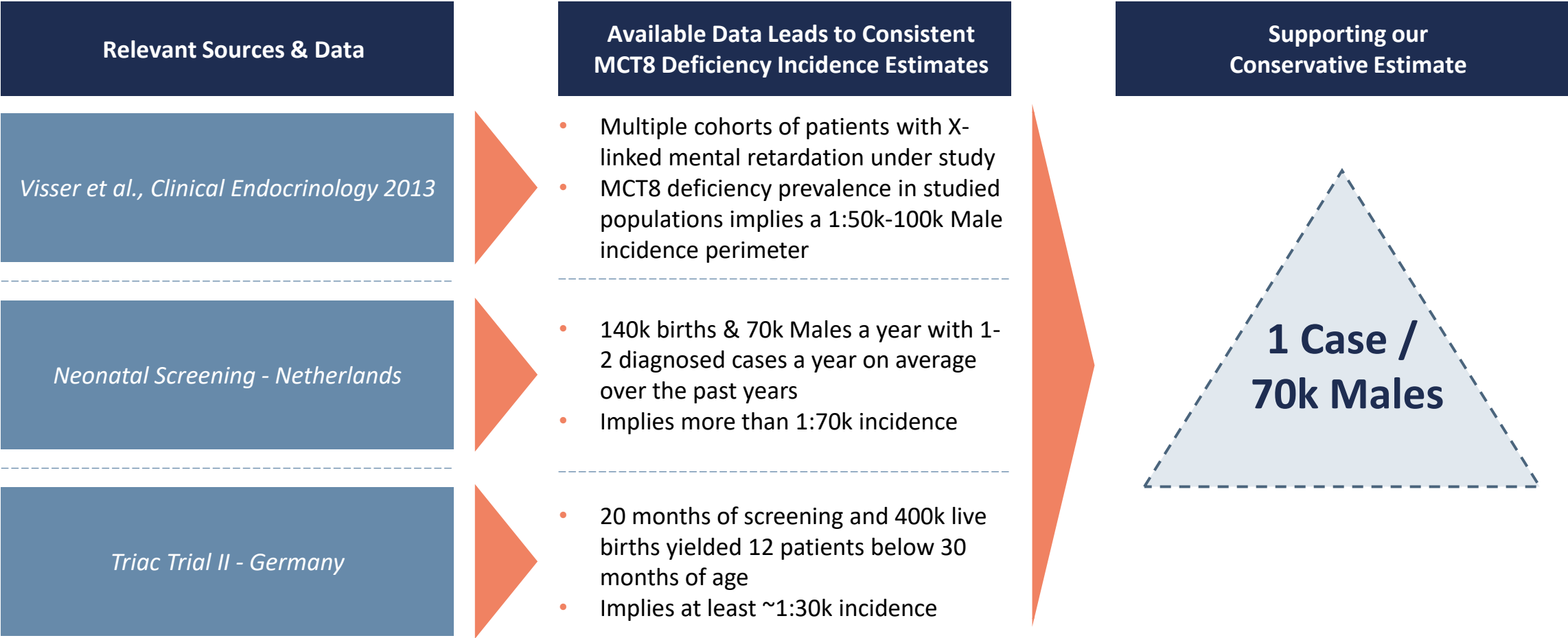
Natural history study revealed poor survival with a high prevalence of treatable underlying risk factors

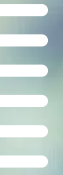
An international, retrospective, multicentre cohort study from 2014-2020 in 151 patients

- 151 patients were enrolled with 73 different MCT8 (SLC16A2) mutations
- Median age at diagnosis was 24.0 months
- 21% patients died; the main causes of mortality were pulmonary infection (six patients or 19%) and sudden death (six patients or 19%)
- Median OS was 35.0 years (95% CI 8.3-61.7)
- Individuals who did not attain head control by age 1.5 years had an increased risk of death compared with patients who did attain head control ($p=0.0041$)
- Patients who were underweight during age 1-3 years had an increased risk for death ($p=0.021$)
- The few motor & cognitive abilities of patients did not improve with age, as evidenced by the absence of significant correlations between biological age and scores on the Gross Motor Function Measure-88 and Bayley Scales of Infant Development III
- Tri-iodothyronine concentrations were above the age-specific upper limit in 96 (95%) of 101 patients and free thyroxine concentrations were below the age-specific lower limit in 94 (89%) of 106 patients. 59 (71%) of 83 patients were underweight. 25 (53%) of 47 patients had elevated systolic blood pressure above the 90th percentile, 34 (76%) of 45 patients had premature atrial contractions, and 20 (31%) of 64 had resting tachycardia
- The most consistent MRI finding was a global delay in myelination, which occurred in 13 (100%) of 13 patients



Multiple sources lead to consistent MCT8 deficiency incidence estimates





2.

Clinical experience with Emcitate

Orphan drug candidate

with clear scientific and mechanistic rationale and established safety profile

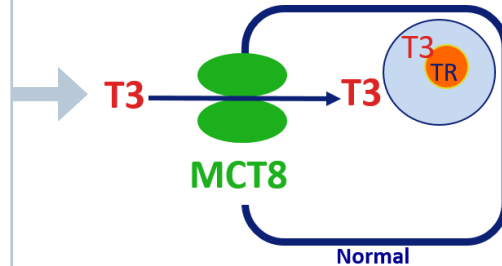


Difference normal MCT8 and deficiency of MCT8

- Thyroid hormone T3 requires transporters such as MCT8 to enter the target cells

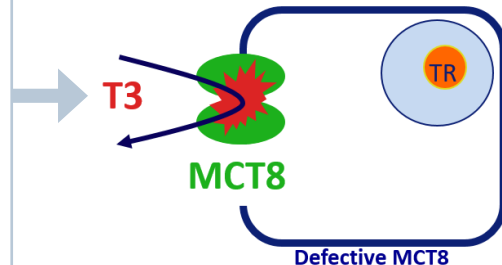
Normal MCT8 ✓

- Functional thyroid gland producing T3
- Production of functional MCT8
- T3 cross cell membrane and enters target cell



Mutated MCT8 ✗

- Functional thyroid gland producing T3
- Absence or loss of function of MCT8 on the cell surface
- T3 cannot cross cell membrane and fails to enter cells

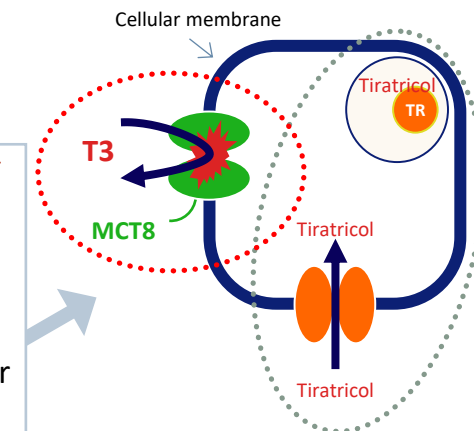


Emcitate (tiratricol) – Addressing MCT8 deficiency

- Tiratricol is a small molecule thyroid hormone T3 analogue
- Unlike T3, tiratricol can cross cellular membranes without a functional MCT8 transporter
- Tiratricol can bypass the problem in patients with MCT8 deficiency, enter MCT8 deficient cells and restore thyroid hormone signalling
- Experience from 40 years on the French market in a different indication, owned and controlled by the Company

Emcitate in action

Without a functioning MCT8, T3 is unable to be transported across the cell membrane to enter the target cell ✗



Emcitate can enter the cell without MCT8 and restore thyroid hormone signaling ✓

Emcitate® Overview

Lead candidate for addressing MCT8 deficiency, a condition with high unmet medical need and no available treatment



Clinical

- **Triac Trial I** completed with significant and clinically relevant effects
- **Erasmus Medical Center cohort study confirms long-term efficacy and safety for up to 6 years (2021)**
- **Triac Trial II:** Results announced June 19, 2024. Did not meet primary neurocognitive endpoints. Confirmed the significant and durable reduction of T3 levels in all patients

Regulatory

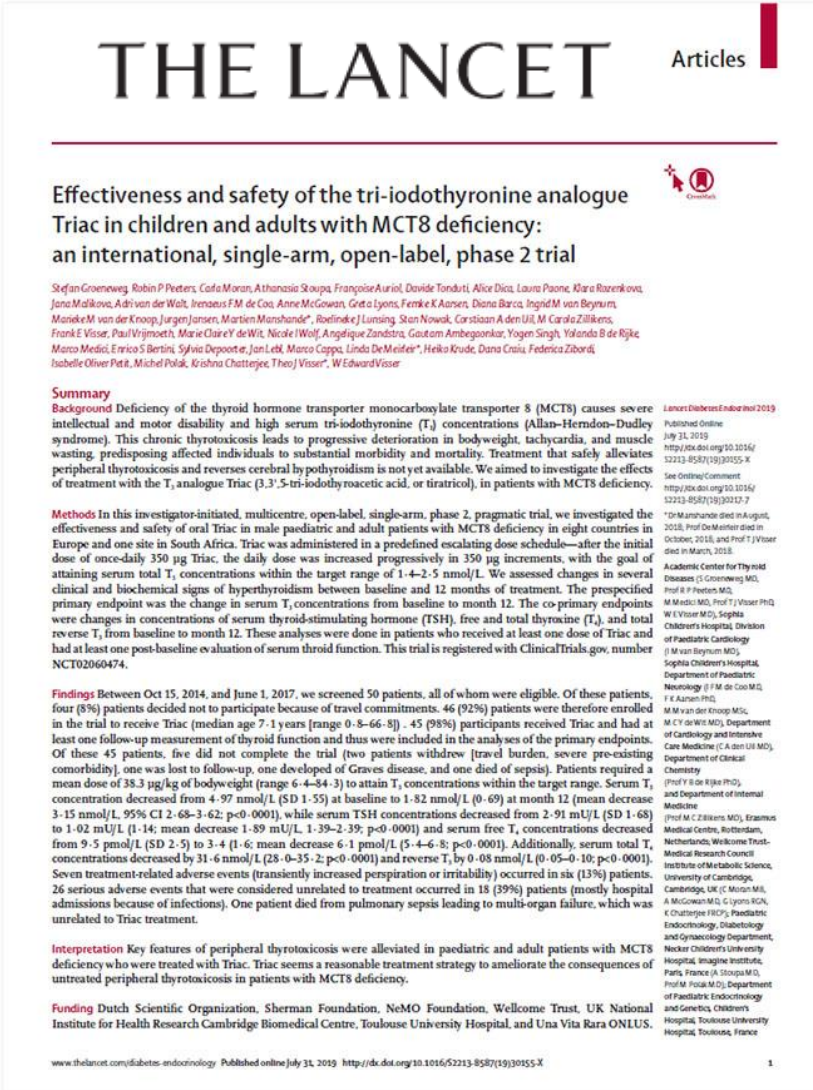
- Orphan drug designation in EU & US, US Rare Pediatric Disease Designation - **eligible for Priority Review Voucher**
- **Fast track designation** granted by FDA
- **MAA submitted to the EMA in October 2023**
- For the US **NDA submission** a 30-day, placebo-controlled study in 16 evaluable patients is being conducted to verify the results on T3 levels seen in previous clinical trials and publications

Commercial

- Incidence 1:70k males, no sponsor-initiated trials ongoing in MCT8 deficiency
- Analogue orphan drugs priced at premium
- **Launched disease awareness initiatives to support diagnosis of MCT8 deficiency**
- **Over 220 patients** are being treated with *Emcitate* in managed access programs, following individual regulatory approvals from national regulatory agencies
- Expected **market exclusivity** is **10 years in EU (ODD), 7 years in US (ODD)**

Overview of completed Phase IIb – Triac Trial I

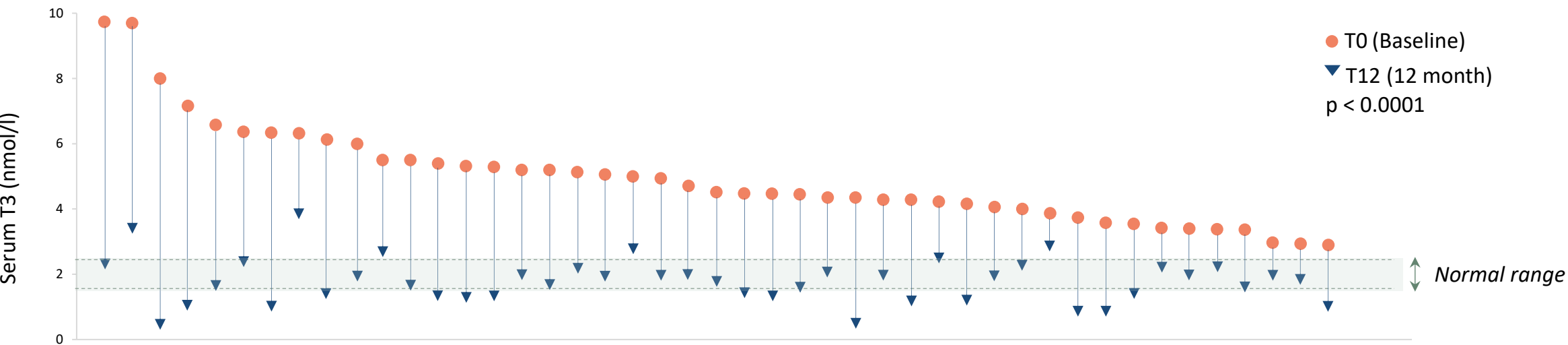
Primary objective and results	<ul style="list-style-type: none">Evaluate the efficacy and safety of oral administration of tiratricol in male patients with MCT8 deficiency of all agesHighly significant primary outcome - Change in T3 serum concentrationsSafe and tolerableResults published in <i>The Lancet</i> 2019
Secondary objective and results	<ul style="list-style-type: none">Change in other thyroid hormone function tests, thyrotoxic symptoms and markersSignificant and clinically relevant effects observed across secondary endpoints
Description	<ul style="list-style-type: none">An international, single-arm, open-label, Phase II trialClinicalTrials.gov identifier: NCT02060474
# of patients	<ul style="list-style-type: none">46 MCT8 patients in 9 countries
Timetable	<ul style="list-style-type: none">Initiated in 2014 (first patient in)Completed in 2018



Source: Groeneweg et al, Lancet D&E 2019;

Consistent, clinically relevant and highly significant results

Triac Trial I: Reached target level serum T3 & improvements in clinically relevant outcome measures

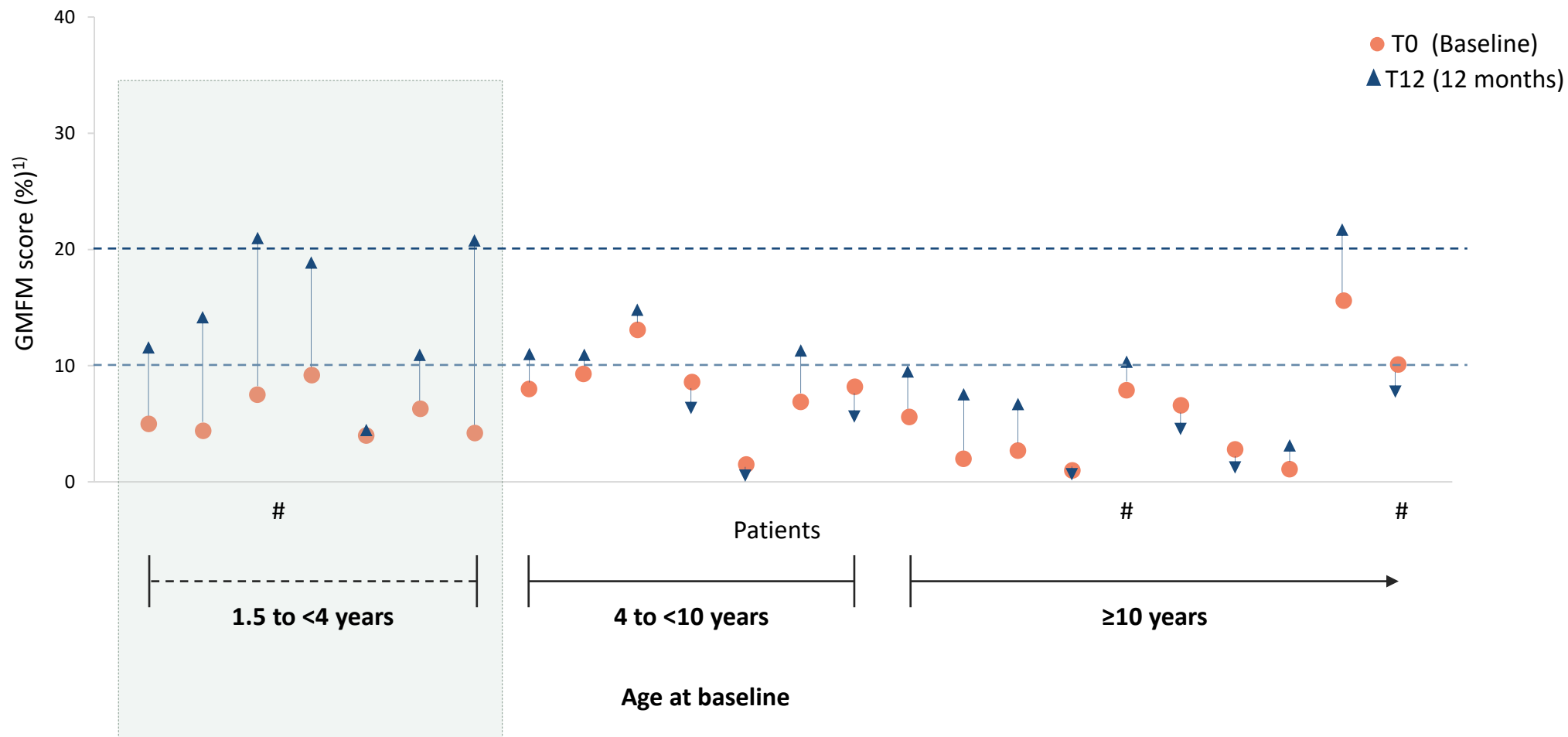


Endpoints	Baseline mean (± SD)	12 months mean (± SD)	Difference in means (95% CI)	p-value
Serum T3 (nmol/L)	4.97 (± 1.55)	1.82 (± 0.69)	-3.15 (-3.62, -2.68)	<0.0001
Weight to age (z score)	-2.98 (± 1.93)	-2.71 (± 1.79)	0.27 (0.03, 0.50)	0.025
Resting heart rate (bpm)	112 (± 23)	104 (± 17)	-9 (-16, -2)	0.01
Mean heart rate 24 h (bpm)	102 (± 14)	97 (± 9)	-5 (-9, -1)	0.012
SHBG (nmol/L)	212 (± 91)	178 (± 76)	-35 (-55, -15)	0.0013
Total cholesterol (mmol/L)	3.2 (± 0.7)	3.4 (± 0.7)	0.2 (0.0, 0.3)	0.056
CK (U/L)	108 (± 90)	161 (± 117)	53 (27, 78)	<0.0001

Source: Groeneweg et al; Lancet D&E 2019

Triac Trial I: Indication of positive effect on neurocognitive development

Triac Trial II did not meet its primary endpoints



Long-term efficacy and safety of Emcitate® in MCT8 deficiency patients

Published in October, 2021

ACCEPTED MANUSCRIPT

Long-term efficacy of T3 analogue Triac in children and adults with MCT8 deficiency: a real-life retrospective cohort study

Ferdy S van Geest, Stefan Groeneweg, Erica L T van den Akker, Iuliu Bacos, Diana Barca, Sjoerd A A van den Berg, Enrico Bertini, Doris Brunner, Nicola Brunetti-Pierri, Marco Cappa ... [Show more](#)

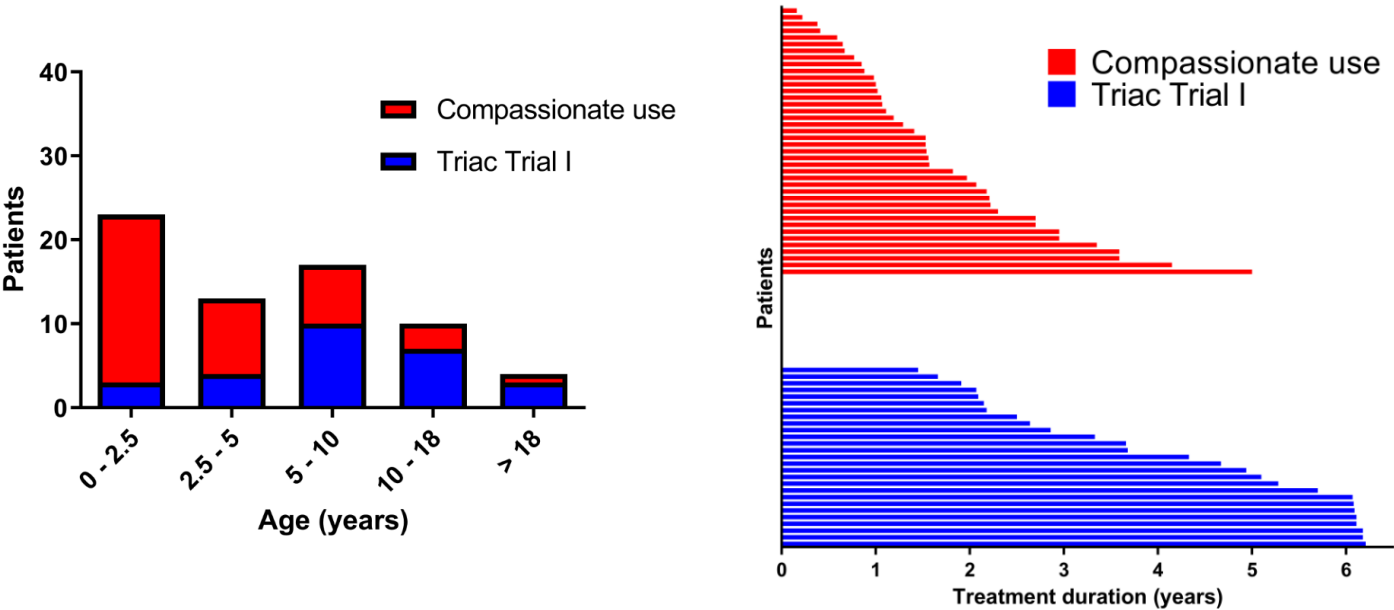
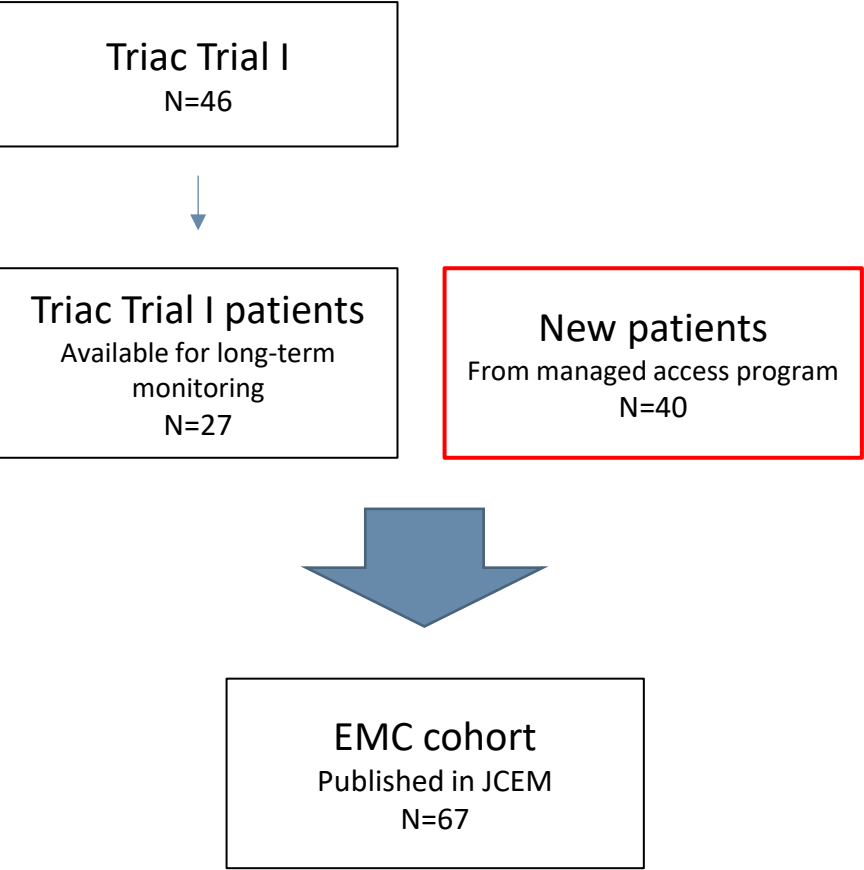
[Author Notes](#)

JCEM THE JOURNAL
OF CLINICAL
ENDOCRINOLOGY
& METABOLISM

- Investigator-initiated real-world cohort study at 33 sites conducted by the Erasmus Medical Center
- Investigated efficacy and safety of *Emcitate* in 67 patients with MCT8 deficiency
 - Median baseline age of 4.6 years (range: 0.5–66 years) and were treated with tiratricol for up to 6 years, with a median of 2.2 years (range 0.2 – 6.2 years)
 - The primary endpoint in the study was the change in serum T3 concentration from baseline to last-available measurement
 - The pre-specified secondary endpoints were key measurements of clinical complications of chronic peripheral thyrotoxicosis

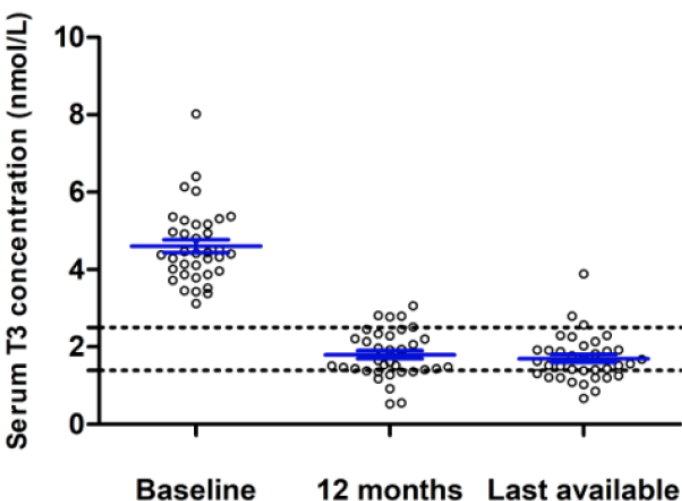
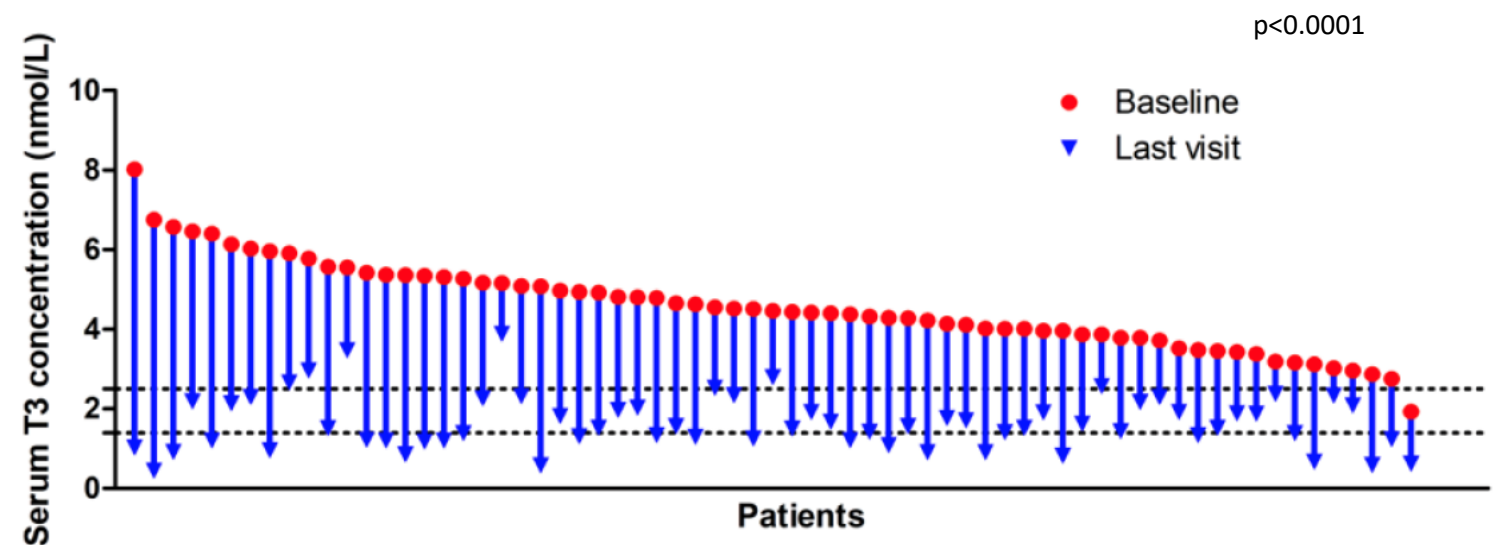
New patient cohort of equal size to the Triac Trial I

Long term follow up, up to >6 years



New cohort confirms primary endpoint results in Triac Trial I

Fast and durable normalization of T3 values in almost all patients

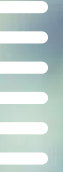


Consistent, clinically relevant and highly significant results across endpoints

- Data confirm the positive results from previous study, Triac Trial I
- Normalization of serum T3 corresponds to improvement in thyroid hormone status in end target tissues
- Beneficial effects are maintained or continue to improve over time, up to six years
- Consistent efficacy seen across key clinical and biochemical parameters that were sustainably alleviated in patients with MCT8 deficiency regardless of age

Table 2: Changes from baseline to last visit in predefined outcomes

	Baseline mean (SD)	Last visit mean (SD)	Mean change (95% CI)	P value
Primary outcome				
T3 (nmol/L; n=67)	4.58 (1.11)	1.66 (0.69)	-2.92 (-3.23 to -2.61)	<0.0001
Secondary outcomes				
<i>Anthropometric parameters and heart rate</i>				
Body weight (kg; n=58)	17.8 (12.1)	23.6 (14.5)	5.7 (4.2 to 7.2)	
Weight-for-age Z score (n=58)	-2.81 (1.94)	-2.64 (1.81)	0.17 (-0.18 to 0.53)	0.3263
Δ Weight-for-age – predicted weight-for-age Z score (n=55)	0.07 (1.83)	0.79 (1.92)	0.72 (0.36 to 1.09)	0.0002
Height (cm; n=44)	101 (21)	116 (23)	15 (12 to 19)	
Height-for-age Z score (n=44)	-1.84 (1.77)	-1.92 (1.51)	-0.09 (-0.50 to 0.32)	0.6705
Δ Height-for-age – predicted height-for-age Z score (n=43)	-0.44 (1.38)	0.14 (1.41)	0.58 (0.12 to 1.05)	0.0139
Weight-for-height Z score (n=44)	-2.02 (2.49)	-1.50 (2.44)	0.52 (-0.35 to 1.39)	0.2358
Heart rate (bpm; n=48)	113 (21)	97 (20)	-17 (-24 to -10)	<0.0001
Heart rate-for-age Z score (n=48)	1.59 (0.89)	0.96 (1.01)	-0.64 (-0.98 to -0.29)	0.0005
<i>Thyroid function tests</i>				
TSH (mU/L; n=62)*	3.32 (2.30)	0.95 (0.73)	-2.38 (-2.98 to -1.77)	<0.0001
Free T4 (pmol/L; n=64)	9.5 (2.3)	3.4 (1.6)	-6.1 (-6.7 to -5.4)	<0.0001
T4 (nmol/L; n=63)	54.2 (11.8)	18.1 (9.8)	-36.1 (-39.5 to -32.7)	<0.0001
<i>Peripheral markers</i>				
Sex hormone-binding globulin (nmol/L; n=48)	245 (99)	209 (92)	-36 (-57 to -16)	0.0008
Creatinine (μmol/L; n=47)	32 (11)	39 (13)	7 (6 to 9)	<0.0001
Creatine kinase (U/L; n=47)*	110 (87)	128 (80)	18 (-8 to 45)	0.2166
All outcomes were assessed in all patients who received Triac treatment longer than the mean time to optimal dose (5.0 months; N=64). Data are mean. Body weight-for-age Z scores were calculated using TNO growth calculator and heart rate-for-age Z scores were calculated using the Boston Z score calculator. Abbreviations: T3=tri-iodothyronine. TSH=thyroid-stimulating hormone. T4=thyroxine. *TSH and creatine kinase concentrations were log-transformed to ensure a normal distribution before paired t tests were done (non-transformed means [SDs] and mean changes [95% CIs] are presented for the sake of interpretability).				



2.

Emcitate[®] - regulatory pathway to submissions in EU and US

Regulatory features of *Emcitate* for MCT8 deficiency



ODD

Orphan drug designation for MCT8 deficiency

Eligibility: Market exclusivity 10y (EU) & 7y (US)

Fast
track

Fast track designation (FDA)

PRV

Rare pediatric disease designation (FDA)

Eligibility: Priority review voucher upon approval*

MAA
NDA

MAA: Submitted in October '23

NDA: Pivotal ReTRIACt study in 16 evaluable patients ongoing



ODD

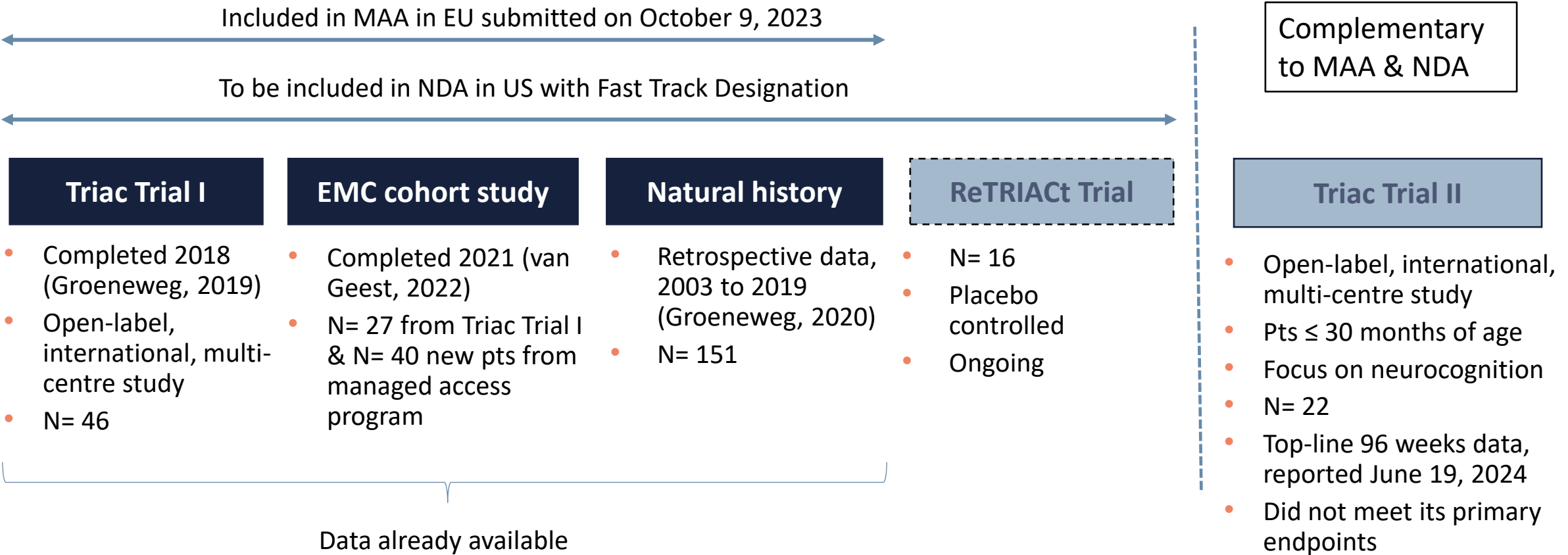
Orphan drug designation for RTH-beta

Eligibility: Market exclusivity for distinct indication

*The voucher may be sold to another sponsor (2021-24 range: ~\$100m-\$158m)

4. Emcitate regulatory pathway in EU and US

The first potential treatment for MCT8 deficiency, an ultra rare genetic disease with high unmet medical need and no available treatment



ReTRIACt: withdrawal of *Emcitate* in males with MCT8 Deficiency

Pivotal randomized placebo-controlled trial for NDA submission



Primary endpoint	<ul style="list-style-type: none">• Proportion of participants who meet the rescue criterion (serum total T3 > ULN) during the 30-day double-blind Randomized Treatment Period
Secondary endpoints	<ul style="list-style-type: none">• Change in cardiovascular variables• Change in serum thyroid hormone variables
Description	<ul style="list-style-type: none">• Double-blind, randomized, multicenter placebo-controlled study• Participants with stable maintenance treatment with <i>Emcitate</i> or treatment naïve patients• Design agreed with FDA; Clinicaltrials.gov identifier: NCT05579327
# of patients	<ul style="list-style-type: none">• At least 16 evaluable patients, > 4 years of age• Patients from Managed Access program and treatment naïve patients
Timetable	<ul style="list-style-type: none">• First patients recruited Q3 2023• Egetis will update the market as soon as recruitment has been completed, and subsequently when top-line results and NDA filing can be expected

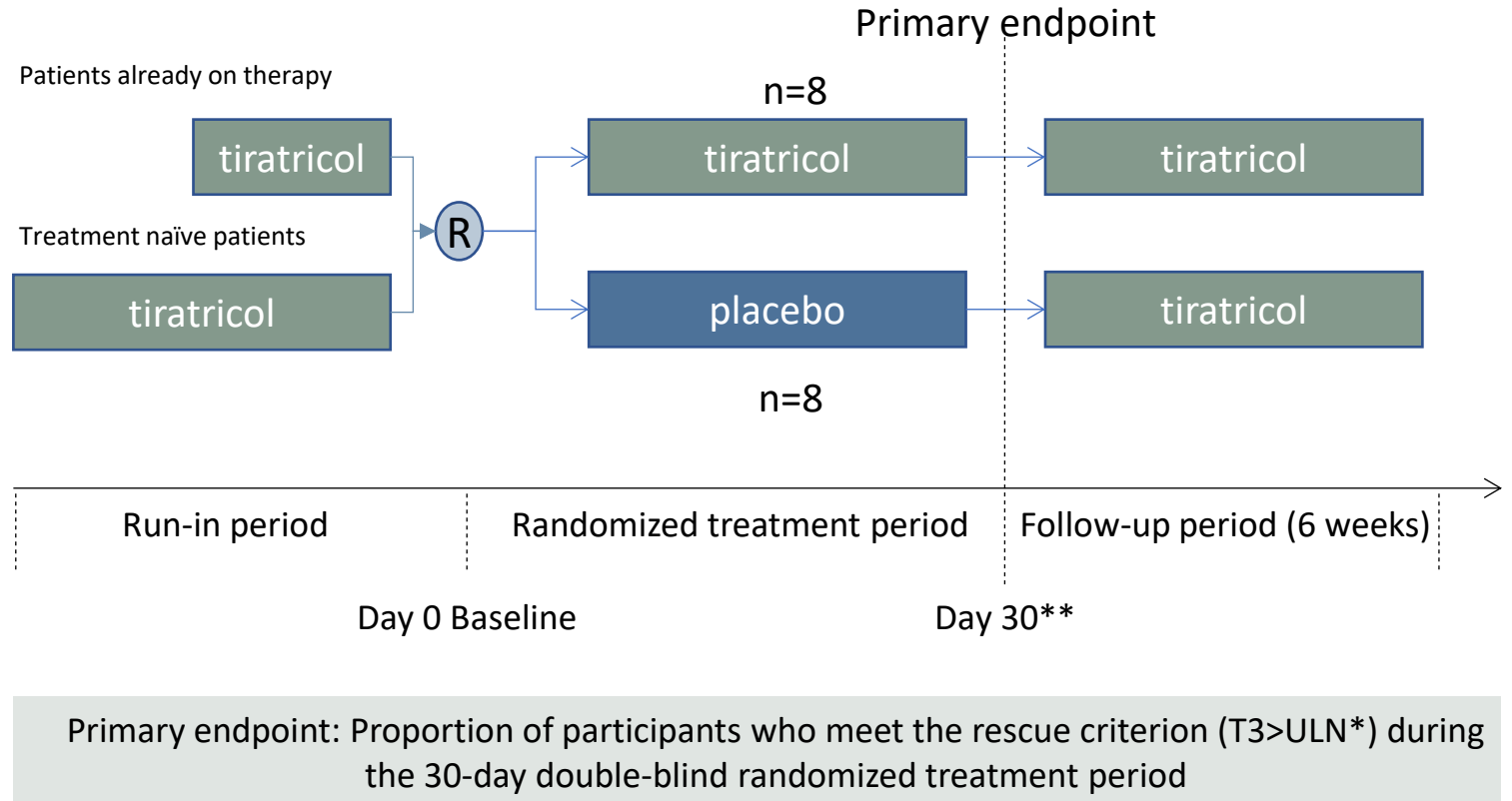


Design of the ReTRIACt clinical trial

Requested by the FDA as pivotal for the New Drug Application in the USA



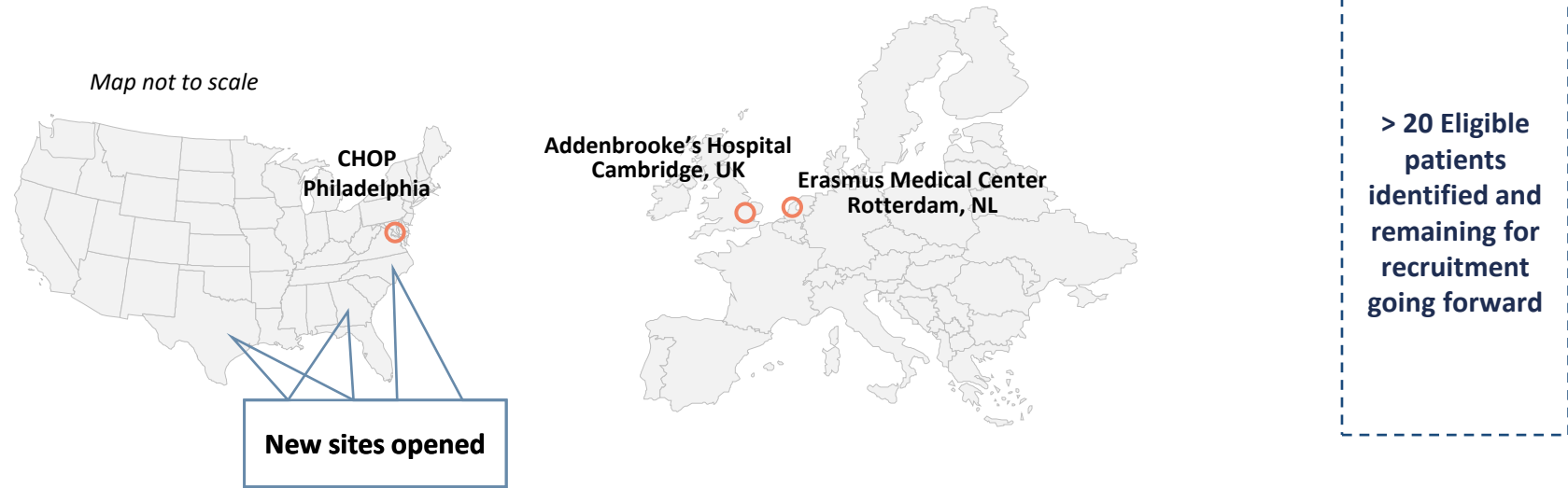
- A 30-day, randomized placebo-controlled withdrawal study in 16 patients
- Design agreed with FDA
- The study allows for inclusion of patients that are already on therapy and patients that are treatment naïve
- Treatment naïve patients require a longer run-in period to stabilize T3 levels around normal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



* ULN: Upper Limit of Normal

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

Current status of ReTRIACt trial (as of Nov 8, 2024)



- 17 patients have been included so far, of which 8 patients have completed the randomized phase.
- 6 sites currently open, including new sites in Georgia, North Carolina and Texas.
- 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 and NDA filing can be expected.

Triac Trial II objective and design:

Triac Trial II was designed to investigate a potential additional benefit on neurocognitive development in 22 patients with MCT8 deficiency below 30 months of age treated with Emcitate® (tiratricol) during 96 weeks

Primary Objective	<ul style="list-style-type: none">Confirm findings from Triac Trial I in youngest age groupImprovement in neurocognitive development as measured by GMFM¹ and BSID-III² compared to natural history controls
Secondary Objective	<ul style="list-style-type: none">Achievement of motor milestones (e.g. hold head, sit independently)Normalization of thyroid hormone function tests and markers of thyrotoxicosis
Description	<ul style="list-style-type: none">Open label, multi-centre trial in very young children with MCT8 deficiencyInternational trial with centres in CZ, DE, NL & USDesign discussed and anchored with EMA and FDAClinicalTrials.gov identifier: NCT02396459
# of Patients	<ul style="list-style-type: none">22 children, 0-30 months of age
Timetable	<ul style="list-style-type: none">Topline 96-week results announced on June 19, 2024The trial did not meet its primary endpoints (please see next slide)Market approval not dependent on Triac Trial II data



1. GMFM: Gross Motor Function Measure
2. BSID: Bayley Scales of Infant and Toddler Development

Triac Trial II Summary



- Triac Trial II results:
 - The numerical improvements versus baseline observed on the primary endpoints of neurocognitive development assessed by the GMFM-88 and BSID-III scales did not show a statistically significant improvement versus historical controls.
 - The trial confirmed the significant and durable reduction of T3 levels in all patients - relevant to alleviate features of thyrotoxicosis in patients with MCT8 deficiency.
 - Well-tolerated safety profile of tiratricol seen in previous clinical studies.
- The Triac trial II is complementary to the data already submitted and validated in the MAA for Emcitate® (tiratricol) for treatment of MCT8 deficiency, based on the benefit of normalization of thyrotoxicosis which has been demonstrated in patients of all ages, as agreed with the EMA. Results from Triac Trial II were included in the response to EMA 120-day list of questions in August 2024.
- The forthcoming NDA in the USA will also be based on the already observed treatment effects on T3 concentrations and the manifestations of chronic thyrotoxicosis together with results from the ongoing ReTRIACt trial, as acknowledged by the FDA.
- The timeline for regulatory review and approval in EU remain unchanged. For the US, as previously communicated, the Company will update the market with regards to timelines for NDA submission as soon as at least 16 evaluable patients have concluded the ongoing ReTRIACt trial.

Pipeline milestones



FDA granted Rare Pediatric Disease designation to Emcitate®

US Rare Pediatric Disease Priority Review Voucher (PRV) provides a ~\$100m opportunity

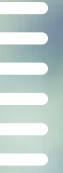


Overview of PRV

- The FDA grants Rare Pediatric Disease designation (RPD) to therapies for serious or life-threatening diseases affecting fewer than 200,000 people in the USA
- Sponsors holding a RPD can apply to receive Priority Review Voucher (PRV) upon approval
- Provides accelerated FDA review of a new drug application for another drug candidate, in any indication, shortening time to market in the US
- The voucher may be sold or transferred to another sponsor
- During 2021-24 PRVs have been sold ranging from \$100m-\$158m

Examples of PRVs sold

Seller	Buyer	Value	Year
Liminal Biosciences	Undisclosed	\$105M	2021
Rhythm Pharmaceuticals	Undisclosed	\$100M	2021
Albireo	Undisclosed	\$105M	2021
Biomarin	Undisclosed	\$110M	2022
BridgeBio	Undisclosed	\$110M	2022
Mallinckrodt	Novartis	\$100M	2022
Marinus Pharmaceuticals	Novo Nordisk	\$110M	2022
Ipsen	Undisclosed	\$158M	2024



2.

Emcitate[®] - Commercial opportunity

Emcite[®] – alleviating patient and societal burden

Aiming to provide value for both patients and society

MCT8 deficiency is a detrimental condition with significant unmet medical need and no approved therapy

Patients

- Median life-expectancy of MCT8 patients is 35 years¹
- Patients underweight for age or without ability to hold head have an even increased risk of premature death

Society

- All MCT8 patients have significant neurocognitive disability from early childhood and typically require constant, life-long supportive care
- A recent study in a condition with similar severity (SMA) estimated total healthcare cost (excluding treatment cost) to USD 138k per patient and year²



Emcite holds potential to become the **first approved therapy** to address the root cause of MCT8 deficiency, restore thyroid hormone signaling and thereby **prevent disease progression**, alleviate symptoms and **prolong lives**

Source: (1) Disease characteristics of MCT8 deficiency: an international, retrospective, multicentre cohort study, Groeneweg et al, The Lancet, 2012; (2) Economic burden of spinal muscular atrophy in the United States: a contemporary assessment, Droege et al, Journal of Medical Economics, 2020;

Emcitate supplied globally in managed access programs

Managed access programs confirm the significant unmet medical need in MCT8 deficiency and the view on how Emcitate address it

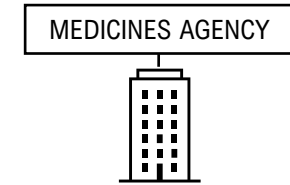
- Managed access programs
 - mechanisms to allow early access to a medicine prior to regulatory marketing approval
 - granted to pharmaceuticals under development for situations with high unmet medical needs and where no available treatment alternatives exist or are suitable
- FDA approved Expanded Access Program - Simplifies Process for Accessing *Emcitate*
- *Emcitate* is being supplied in managed access programs, following individual approval from the national medicines agencies, to
 - Over 220 patients
 - Over 25 countries



Patient

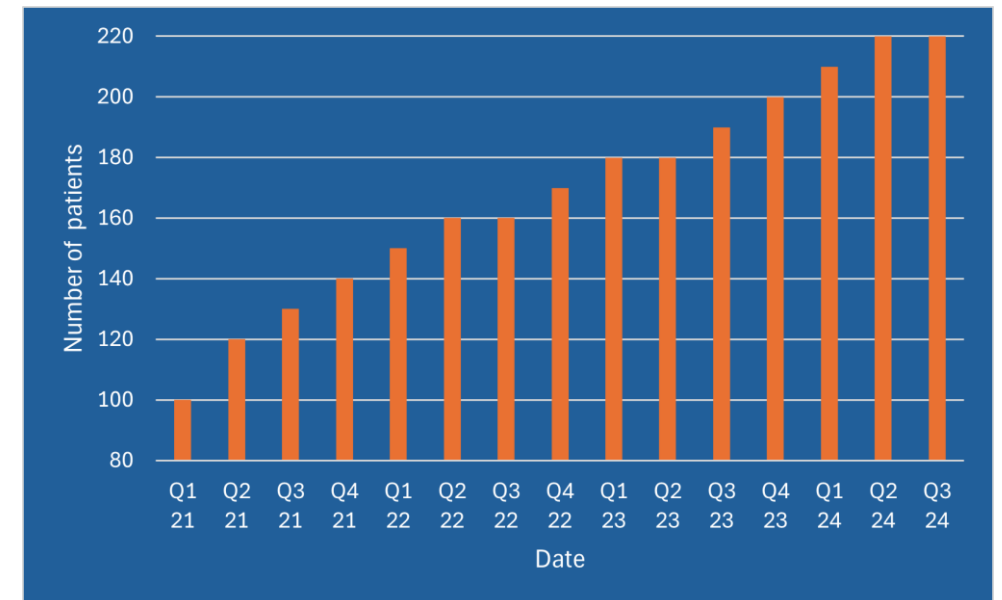


Prescriber



National Approval

Patients Receiving Emcitate in Managed Access Programs



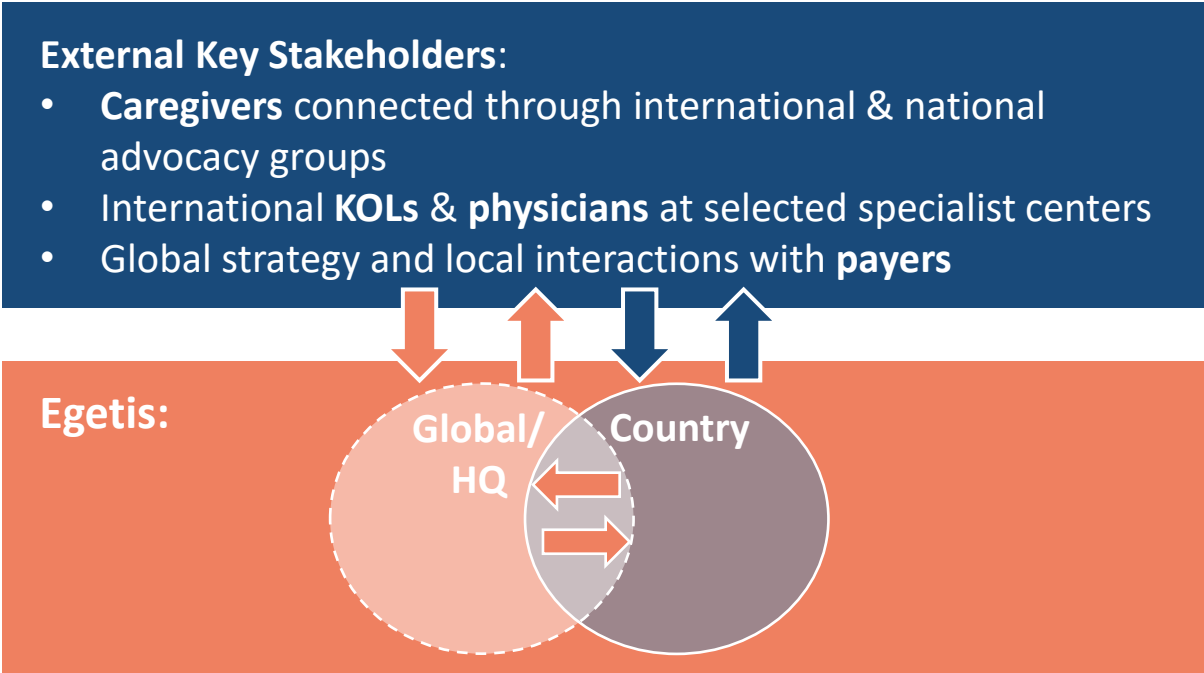
Commercialization possible with lean & agile team

Unique setting for Emcitate in MCT8 deficiency



Seizing opportunity for cost-effective value creation

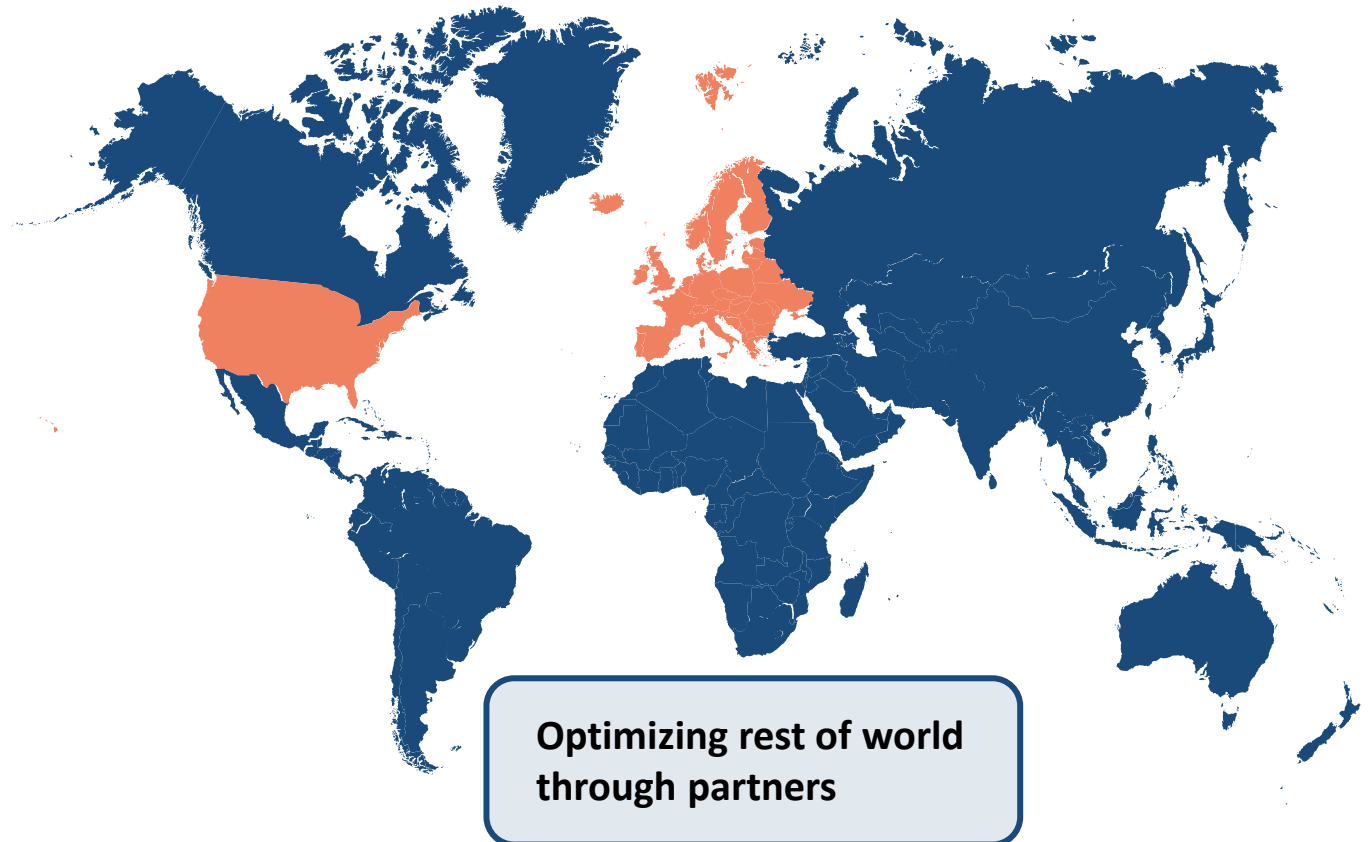
- Targeted stakeholder interactions
- Efficiency gains through global-country team coordination



Egetis commercialization of *Emcitate*

Retaining most value by managing the US & European launches through the Egetis team

To optimize the launch, we will focus our own resources on US and Europe (> 70% of sales for most ultra-orphans)



Stepwise establishing Egetis commercial capabilities

- Preparing for **launch** in US and Europe with an organization of **40-50** employees at time of launch

Building commercial organization to execute on key activities at the right time for launch success



Key projects driven by recognized industry talents recruited to the Egetis Commercial & Medical Affairs Team

– Leadership team brings launch skills and best practices from in total 100+ years at international companies



Henrik Krook, SE
VP, Commercial Operations



Anny Bedard, US
President Egetis North America



Marianne Berrens-Peijnenburg, NL
Medical Affairs



Azza Trad, FR
GM France



Nadia Georges, CH
Global Head,
Market Access & Pricing



Nigel Nicholls, UK
Global Patient Advocacy Director
& GM UK & Northern Europe



Peter Verwaijen, NL
Global Head Marketing & Brand
Strategy, GM Benelux & Iberia



Raymond Francot, CH
GM for DE, AT, CH &
Central & Eastern Europe



Focusing on Critical Areas for Launch Success



Aiming to Improve the Lives of MCT8 Deficiency Patients and their Caregivers

IDENTIFY PATIENTS

Boost disease awareness, educate on disease*, diagnosis and newborn screening



ENSURE ACCESS

Preparing for broad access to Emcitate as soon as possible after marketing authorization

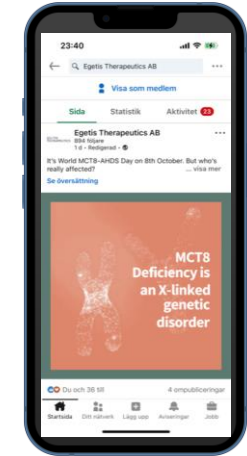
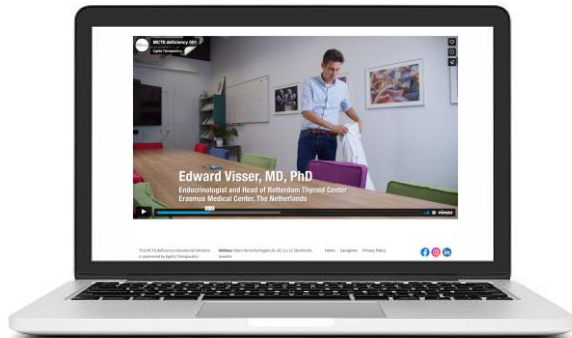


*Emcitate promotion will start at the time of marketing authorization (in line with legislations). Before that, external initiatives are focused on MCT8 deficiency.

Enabling patient identification through disease awareness

Increasing number of previously un- / misdiagnosed and treatment naïve patients are being identified

mct8deficiency.com



DISEASE AWARENESS AND EDUCATION

- Focus on enabling early and accurate diagnosis
- ↑ number of physicians who
 - Are aware of MCT8 deficiency
 - Can diagnose
 - Understands how to manage MCT8 deficiency

COLLABORATION WITH PAGs & KOLs

- KOL engagements and peer-to-peer education through national specialist societies
- International & national patient advocacy groups



EXHIBIT AT SCIENTIFIC/MEDICAL CONFERENCES

- Euro Paediatric Neuro. Society
- European Thyroid Association
- European Society of Paediatric Endocrinology
- International Child Neurology Congress
- American Thyroid Association
- And more...

OPTIMUM CHANNEL MIX FOR MAXIMUM REACH

- MCT8deficiency.com
- Instagram and Facebook
- Mailing campaigns to HCPs
- Social media and video for MCT8-AHDS day (Oct 8th)
- Congresses and F2F interactions
- Publications

Aiming for broad access at optimal reimbursed price



LAUNCH VISION

Everyone who can benefit from treatment, gets access to reimbursed Emcitate asap after regulatory approval



CRITICAL SUCCESS FACTORS

Fast and accurate diagnosis of affected patients

Payer support for reimbursement at optimal price



EXAMPLES OF KEY ENABLERS

Understand & address needs in MCT8 deficiency

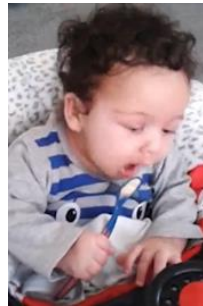
Disease awareness & diagnosis initiatives

Demonstrate & communicate *Emcitate* value

Developing a compelling *Emcitate* clinical and economic value proposition to secure reimbursement & access

Key for payer assessments to describe unmet need & quantify burden of disease

- A significant unmet medical need, addressed by the *Emcitate* clinical development program
- In addition, generating data for payers to answer the question “*What is the burden of MCT8 deficiency for patients, their caregivers & the healthcare?*”



Vignette study

Physician quantifying the impact of the disease on the patients' quality of life



Caregiver study

Focused on costs & quality of life for parents/caregivers



Healthcare resource use study

Describing healthcare resource use for the management of patients

Our Expanded Access Program is a vital step on our path to commercialization



Tiratricol (Emcitate) Expanded Access Program sites



A significant asset to both the patients and Egetis launch readiness

- Provided early and sustainable access to therapy
- Expose physicians to Emcitate prior to commercial approval
- Collect real world data to support payer and regulatory communications

Patient-centric implementation

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

<https://www.clinicaltrials.gov/study/NCT05911399>

Preparing for broad access at an optimized price



Analogue selection criteria

- | | |
|-------------------------|----------------------------------|
| ✓ Rarity (ultra-orphan) | ✓ Life-long treatment |
| ✓ Paediatric | ✓ Life threatening, debilitating |
| ✓ No treatment options | |

US payer analogues

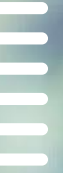
Product	Disease	US gross annual treatment cost
Exondys® anti-sense oligonucleotide	Duchenne Muscular Dystrophy (13% of population)	\$750k
Ravicti® small molecule	Urea Cycle Disorders	\$750K
Oxlumo® iRNA	Primary Hyperoxaluria	\$500k
Brineura® recombinant enzyme	CLN2	\$750k

Most MCT8 deficiency patients will be eligible for government insurance

- Predominantly government payer mix

Exclusive distribution model through specialty pharmacy

- Expertise in negotiating with insurers
- Personalized patient access assistance
- Maintaining control of net price



3.

Paracetamol/Acetaminophen overdose and clinical experience with Aladote

* In-house development of *Aladote* has been parked until *Emcitate* MCT8 deficiency submissions have been completed

Paracetamol/acetaminophen poisoning

– *no adequate treatment for increased-risk patients*



What is paracetamol/acetaminophen poisoning?

- Minimum toxic dose of paracetamol/acetaminophen in adults is only **7.5g**
- Risk factors include malnutrition, alcoholism and consumption of other medications
- Paracetamol/acetaminophen poisoning can lead to **acute liver failure, liver transplant or death**

How many does it affect?

- **19 billion** units of paracetamol /acetaminophen packages are sold in the US alone every year
- **>175,000 patients hospitalised globally per annum** driven by 89,000 cases/year of paracetamol overdose in the US and 105,000 cases/year in the UK (~ 50% hospitalised)
- ~50% of paracetamol overdose cases are unintentional

Why is current treatment inadequate?

- Efficacy of current NAC (N-acetylcysteine) treatment decreases with time
- Approximately **25% of patients are late arrivals** to hospitals (>8h) – late arrivals are **at increased risk**
- There is **no effective treatment option for patients at increased risk**

A new standard of care is needed

- **Aladote®** aims to become **a new standard of care** for patients with increased risk for liver injury in combination with NAC

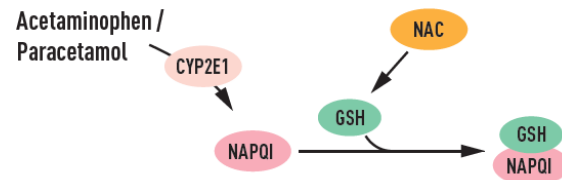
Orphan drug candidate

with clear scientific and mechanistic rationale

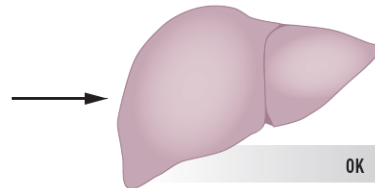
Early presenters (<8h)

NAC treatment effective against liver injury

- Liver glutathione (GSH) replenished by NAC, toxic NAPQI metabolite excreted as GSH conjugate



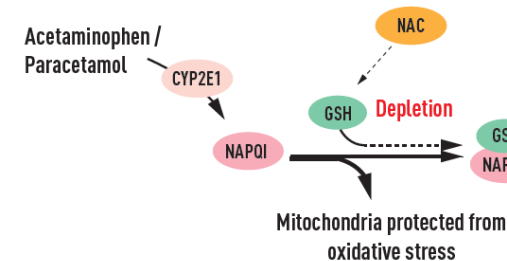
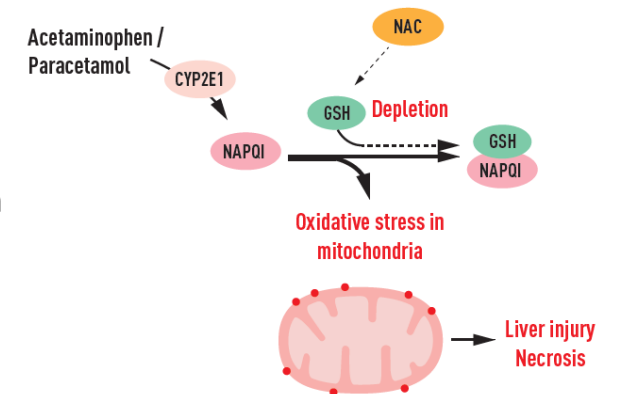
- In most cases NAC effectively prevents liver injury i.e. limited need for Aladote®



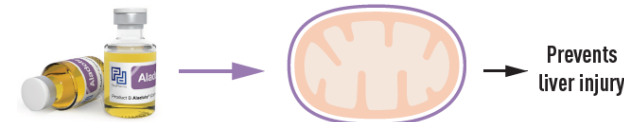
Late presenters (>8h) are at increased-risk for liver injury

NAC treatment + Aladote® to prevent liver injury

- Under NAC treatment alone** liver GSH stores depleted by the toxic NAPQI metabolite → **oxidative stress, mitochondrial dysfunction and liver injury (necrosis)**



- Aladote®** (calmangafodipir) prevents ROS and RNS formation, restores mitochondrial energy production and **prevents liver injury**



Reactive nitrogen species (RNS), Reactive Oxygen Species (ROS)

Overview of completed Phase Ib/Ila

Primary objective and results	<ul style="list-style-type: none">Met primary endpoint of safety tolerability in the combination of Aladote® and NACResults presented at the 58th Annual Meeting of the Society of Toxicology, EASL ILC in April, Vienna and published in Lancet's journal EBioMedicine in 2019Presented at, American College of Medical Toxicology (ACMT) and Society of Toxicology (SOT), as novel emerging treatments for acetaminophen/paracetamol toxicity in 2021
Secondary objectives and results	<ul style="list-style-type: none">Measurements of Alanine transaminase (ALT), international normalised ratio (INR), keratin-18, caspase-cleaved keratin-18 (cck18) and microRNA-122 (mir122) and glutamate dehydrogenase (GLDH) indicates that Aladote® reduce liver injury
Description	<ul style="list-style-type: none">An open label, rising-dose, randomized study exploring safety and tolerability of Aladote® co-treatment with NACClinicalTrials.gov identifier: NCT03177395
# of patients	<ul style="list-style-type: none">Single ascending dose study in 3 dosing cohorts of 8 patients (N=24) as add-on to NAC regime
Timetable	<ul style="list-style-type: none">Initiated in June 2017 (first patient in)Completed in September 2018

EBioMedicine 46 (2019) 423–430

Contents lists available at ScienceDirect

EBioMedicine

journal homepage: www.ebiomedicine.com

EBioMedicine

Published by THE LANCET

Principal results of a randomised open label exploratory, safety and tolerability study with calmagafodipir in patients treated with a 12 h regimen of N-acetylcysteine for paracetamol overdose (POP trial)

Emma E. Morrison^a, Katherine Oatey^b, Bernadette Gallagher^c, Julia Grahamslaw^c, Rachel O'Brien^c, Polly Black^c, Wilina Oosthuizen^a, Robert J. Lee^b, Christopher J. Weir^b, Dennis Henriksen^d, James W. Dear^{a,*}, On behalf of the POP Trial Investigators¹

^a Pharmacology, Therapeutics and Toxicology Unit, Centre for Cardiovascular Science, University of Edinburgh, UK
^b Edinburgh Clinical Trial Unit, UK
^c Emergency Medicine Research Group, Royal Infirmary of Edinburgh, UK
^d MedPharma AB, Stockholm, Sweden

EASL | THE INTERNATIONAL LIVER CONGRESS™ 2019

WATCH CONGRESS SESSIONS

CALMANGAFODIPIR MAY REDUCE LIVER INJURY AFTER PARACETAMOL OVERDOSE: FINAL RESULTS FROM THE POP TRIAL

ILC 2019: Phase 1 study demonstrates that superoxide dismutase mimetic, calmagafodipir, is well tolerated and may reduce liver injury after paracetamol overdose

12 April 2019, Vienna, Austria

EASL (EUROPEAN ASSOCIATION FOR THE STUDY OF THE LIVER)

Final results from a Phase 1 study suggest that the novel superoxide dismutase mimetic, calmagafodipir, is well tolerated and may reduce liver injury after paracetamol overdose. The study which was presented at the

EASL | THE INTERNATIONAL LIVER CONGRESS™ 2019

PRESS RELEASE

Positive proof-of-principle Phase Ib/IIa results

Indicates that Aladote may reduce liver injury

Safety & tolerability

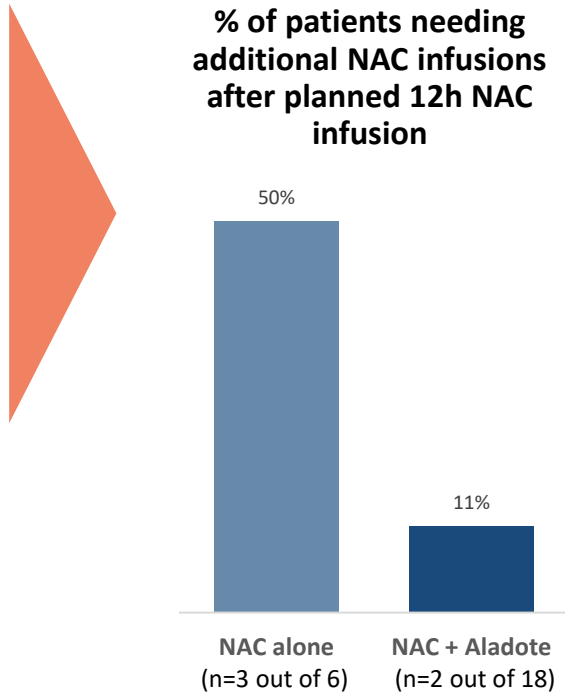
Event	NAC alone	NAC + 2 μ mol/kg Aladote	NAC + 5 μ mol/kg Aladote	NAC + 10 μ mol/kg Aladote
Any AE	6 (100%)	6 (100%)	6 (100%)	6 (100%)
Any SAE	2 (33%)	4 (67%)	2 (33%)	3 (50%)
SAE Starting within 7 days	1 (17%)	1 (17%)	1 (17%)	2 (33%)

- Met primary endpoint of safety tolerability in the combination of Aladote® and NAC
- No AE or SAE probably or definitely related to Aladote®

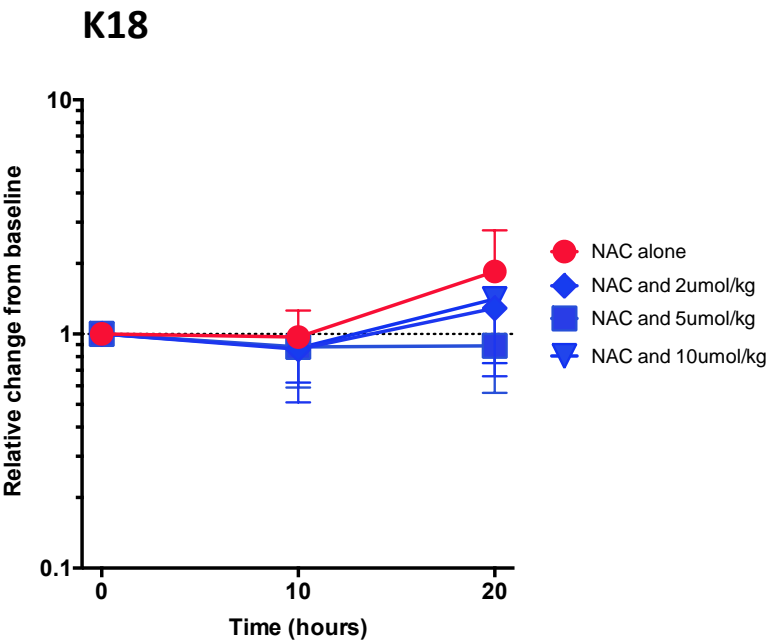
Liver injury – ALT¹ pre-defined secondary outcome

Event	NAC alone	NAC + 2 μ mol/kg Aladote	NAC + 5 μ mol/kg Aladote	NAC + 10 μ mol/kg Aladote
50% ALT increase	2 (33%)	0 (0%)	0 (0%)	1 (17%)
100% ALT increase	1 (17%)	0 (0%)	0 (0%)	1 (17%)
ALT >100 U/L at 10 hours	2 (33%)	0 (0%)	0 (0%)	0 (0%)
ALT >100 U/L at 20 hours	2 (33%)	0 (0%)	0 (0%)	0 (0%)

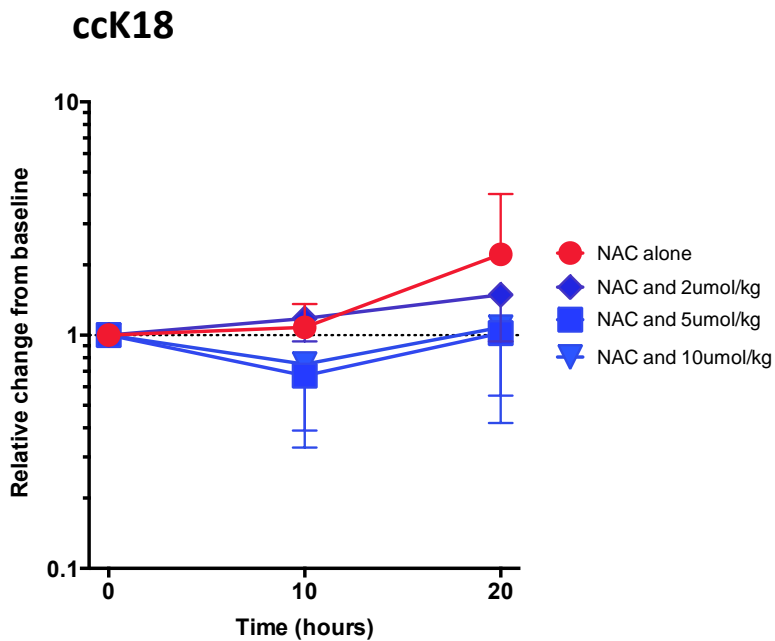
- ALT >100 U/L is the indication to stay in hospital



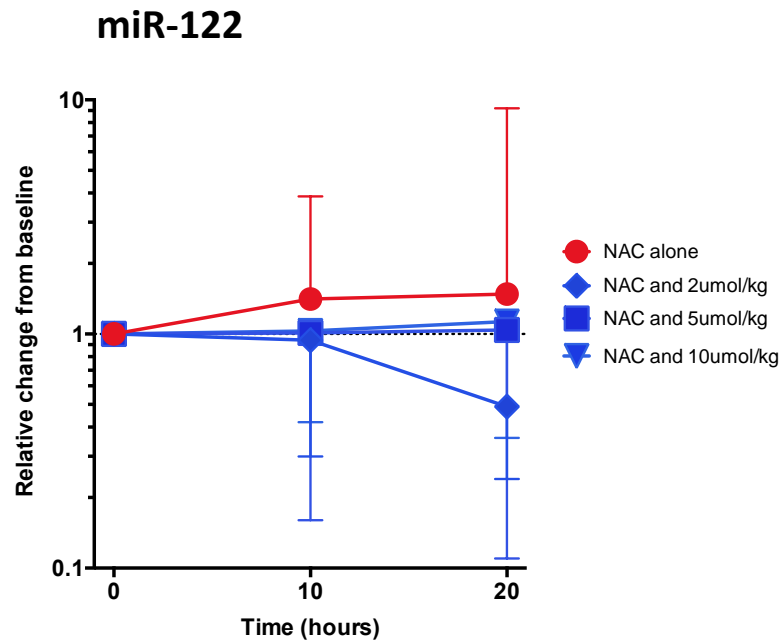
Aladote[®] demonstrates consistent results of reduced liver injury as measured by exploratory biomarkers



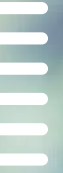
K18 is a measure of cell death and correlate with peak ALT activity during the hospital stay



ccK18, is a measure of cell death and correlate with peak ALT activity during the hospital stay



miR-122 is a liver specific early marker (micro-RNA) for acute liver injury which predicts a rise in ALT activity following paracetamol overdose



3.

*Aladote[®] - Regulatory pathway to submissions in EU and US**

* In-house development of *Aladote* has been parked until *Emcitate* MCT8 deficiency submissions have been completed

ALBATROSS: Phase IIb/III study for US/EU regulatory submission*



Patient population	<ul style="list-style-type: none">• Patients who have overdosed on paracetamol with increased risk of liver damage due to late arrival at hospital (> 8h) who need treatment with NAC
NAC regimen	<ul style="list-style-type: none">• Approved 21 hours NAC regimen
Treatment groups	<ul style="list-style-type: none">• 4 groups in combination with NAC: <i>Aladote</i> high dose; <i>Aladote</i> middle dose; <i>Aladote</i> low dose; Placebo
Initiation of active treatment	<ul style="list-style-type: none">• IV (bolus) as soon as possible after randomization and after starting NAC treatment (but no later than 4 hours after starting NAC treatment)
Interim analysis	<ul style="list-style-type: none">• Interim analysis after 35 patients per treatment group, which includes a futility analysis, dose selection and analysis of continued study size (number of patients)
Study size	<ul style="list-style-type: none">• 250 patients planned
Efficacy endpoints	<ul style="list-style-type: none">• Primary: Combination of ALT and INR• Number (%) of patients who need extended NAC treatment after 21 hours• Length of hospital stay• Explorative biomarkers: K18, miR-122 and GLDH
Study countries	<ul style="list-style-type: none">• EU, UK and USA

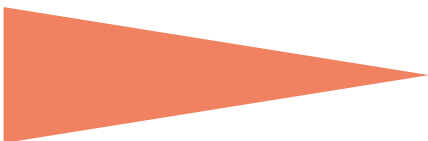


*Study parked until *Emcitate* submissions have been completed

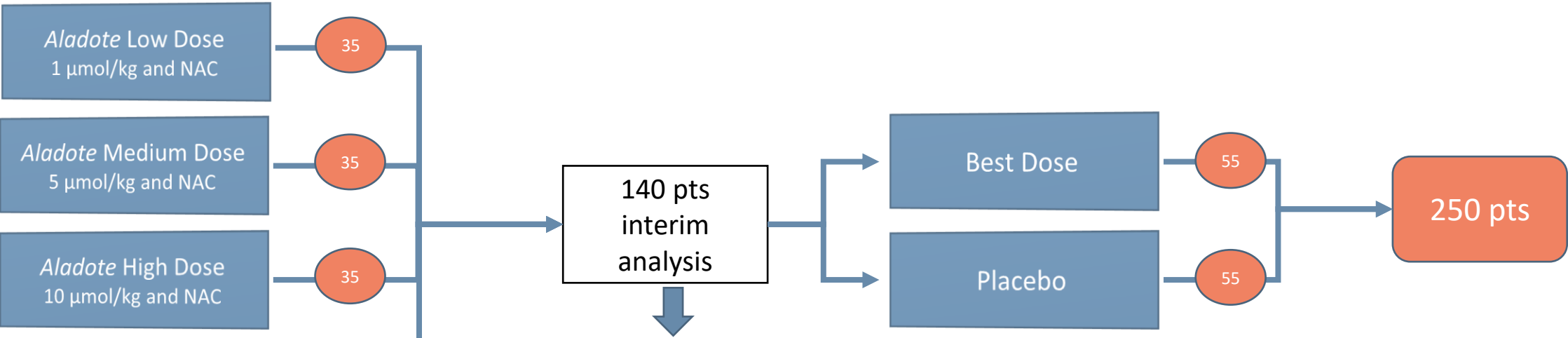
ALBATROSS: Aladote Phase IIb/III study design



Seamless Phase IIb/III design



Based on the acetaminophen/paracetamol levels eligible patients will be randomised in a 1:1:1:1 ratio to one of the 4 treatment arms in combination with NAC:



- Stop the study for futility; or
- Continue the study with the most effective of the 3 doses and placebo up to the preplanned sample size; or
- Increase the sample size in the arm with the most effective dose and the placebo arm to boost the power

Primary endpoint:
Patients w/o hepatic injury
INR ≤ 1.3 and ALT ≤ 50 or
INR ≤ 1.3 and ALT > 50 but not
increased by >10% up to 20h

Aladote clinical development timelines

- ✓ Orphan Drug Designation EU
- ✓ CTA pivotal Phase IIb/III study

2022

tbc

tbc

tbc

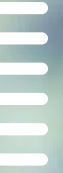
- Start pivotal Phase IIb/III study (after *Emcitate* submissions have been completed)

- Interim analysis
- Recruitment completed and topline results

- Regulatory submissions Europe/US
- Europe/US approvals and launch
- Regulatory submissions ROW

Orphan drug designation in US and EU
Composition of matter patent expires in 2032
Method of use patent until 2037





3.

Aladote[®] - Commercial opportunity

Aladote– alleviating patient and societal burden

Aiming to provide value for both patients and society



POD is a life threatening condition with remaining medical needs

Patients

- POD (paracetamol/acetaminophen overdose) can lead to acute liver failure, liver transplant or death
- In US and UK together, yearly > 500 deaths due to POD and more people registered for liver transplantation

Society

- In the US the annual cost has been estimated at > \$1bn to treat patients with POD¹
- The POD Emergency Department and inpatient cost is approximately USD 13-40k¹
- The average POD inpatient length of stay is 3.1 days with a variance of +4.4 days for the most severe cases¹
- US liver transplant costs USD 125-473k¹



With **Aladote**, the ambition is to **reduce hepatic injury** of POD and thereby contribute to **fewer hospitalization days, prevent need** for liver transplantation and **increase survival**

Source;; (1) Adapted from: Altyar A. Clinical and economic characteristics of emergency department visits due to acetaminophen toxicity in the USA BMJ Open 2015;5;

Commercialisation of *Aladote* for high-risk POD patients

Very cost-effective since possible to launch through members of Emcitate team



Favorable conditions for launch success

Addressing unmet medical need



Leading KOL support



Centralized, **focused target groups** of **specialists** eager to improve care



Treatment choice **highly protocol driven**



No competition

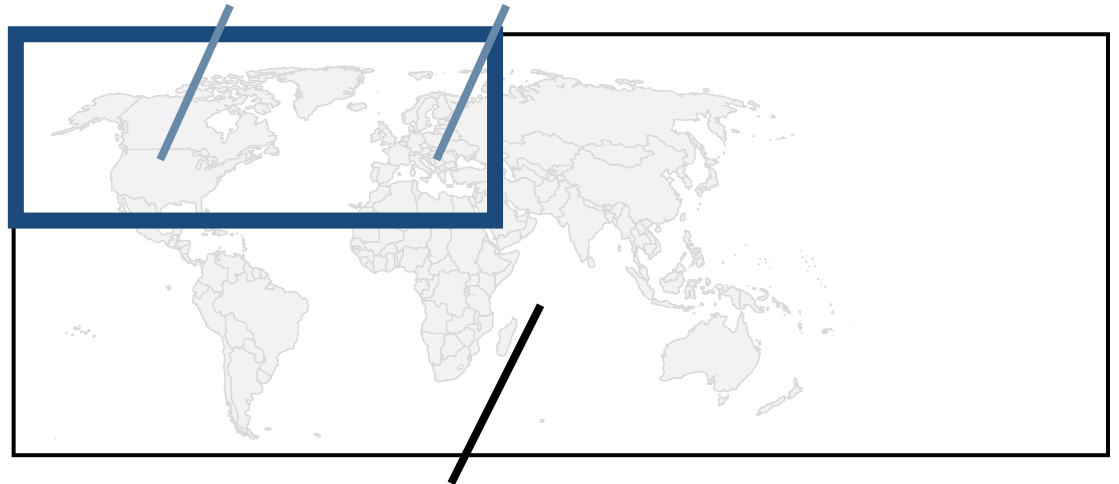


Addressing life-threatening condition

- Analogue antidotes priced at \$3.5k – 50k
- National emergency hospital stocking guidelines gives opportunity to work through **small team** and still ensure **rapid sales uptake**

Hospitalized POD patients per year

US: > 40,000 patients Europe: > 140,000* patients*



Commercialization in rest of world managed through partners

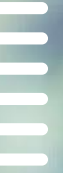
*Annual number of POD (paracetamol/acetaminophen overdose) cases hospitalized and receiving i.v. antidote (NAC currently the only option), 25% late arrivals (>8h)

Analogue antidotes priced at \$ 3.5k – 50k

National emergency hospital stocking guidelines - opportunity for rapid market penetration

- Various antidotes, e.g. vs. drug overdosing, metal poisoning, snake bites and reversal of anticoagulant treatment effects
- Limit morbidity/mortality when used within appropriate time
- National recommendations for stocking of antidotes at hospitals providing emergency care
 - For getting payer/formulary committee acceptance to be stocked, antidotes are in general priced lower than traditional orphan drugs, despite often having orphan status
 - Getting included provides great opportunity for rapid market penetration
 - Praxbind stocked in 3,200 US hospitals < 3 years from launch
 - Andexxa sales \$112mn in US alone second year on market
- Analogue antidotes for comparable settings as Aladote have global average costs of \$ 3.5k – 50k per treatment

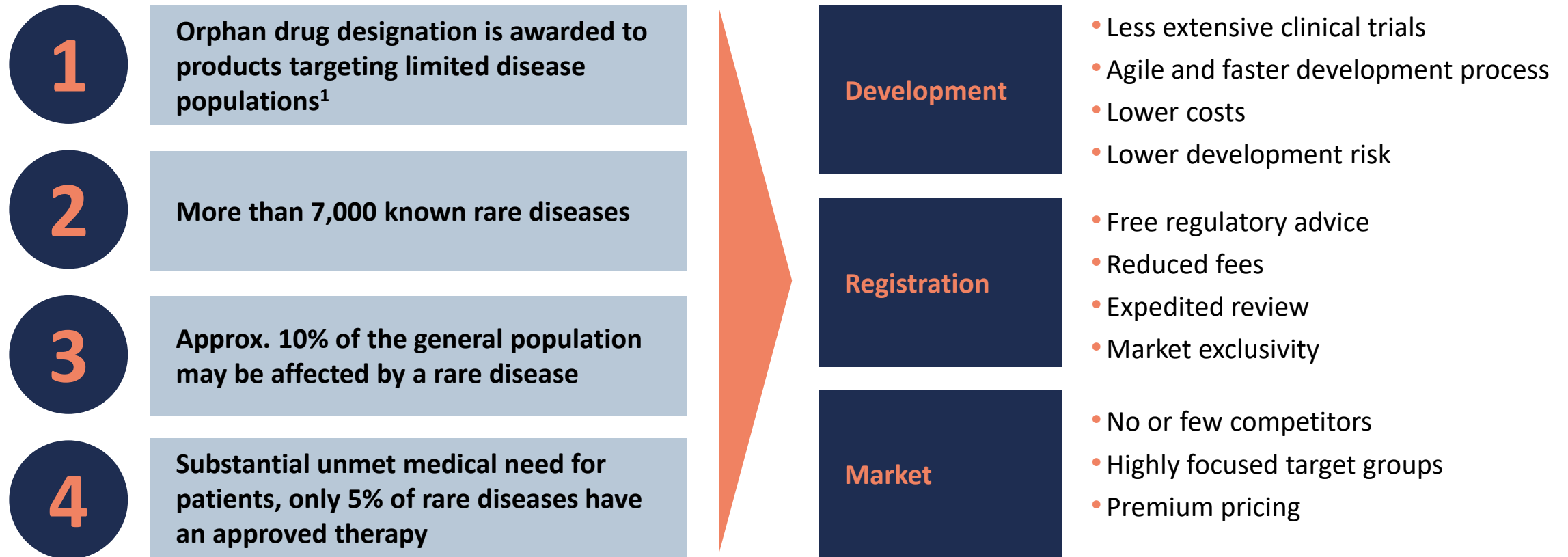
	Naloxone hydrochloride	Praxbind	Andexxa/On dexxya	Aladote (target profile)
Year of first approval	1971	2015	2018	NA
Poisoning indication	Opioid toxicity	Reversal of anticoagulant effects of the NOAC dabigatran	Reversal of anticoagulant effects of the factor Xa inhibitors apixaban & rivaroxaban	Paracetamol/acetaminophen toxicity
Cost per treatment	Low since generic	\$ 3.5k – 4.5k	\$ 25k – 50k	TBD



4.

The attractiveness of the orphan drug segment

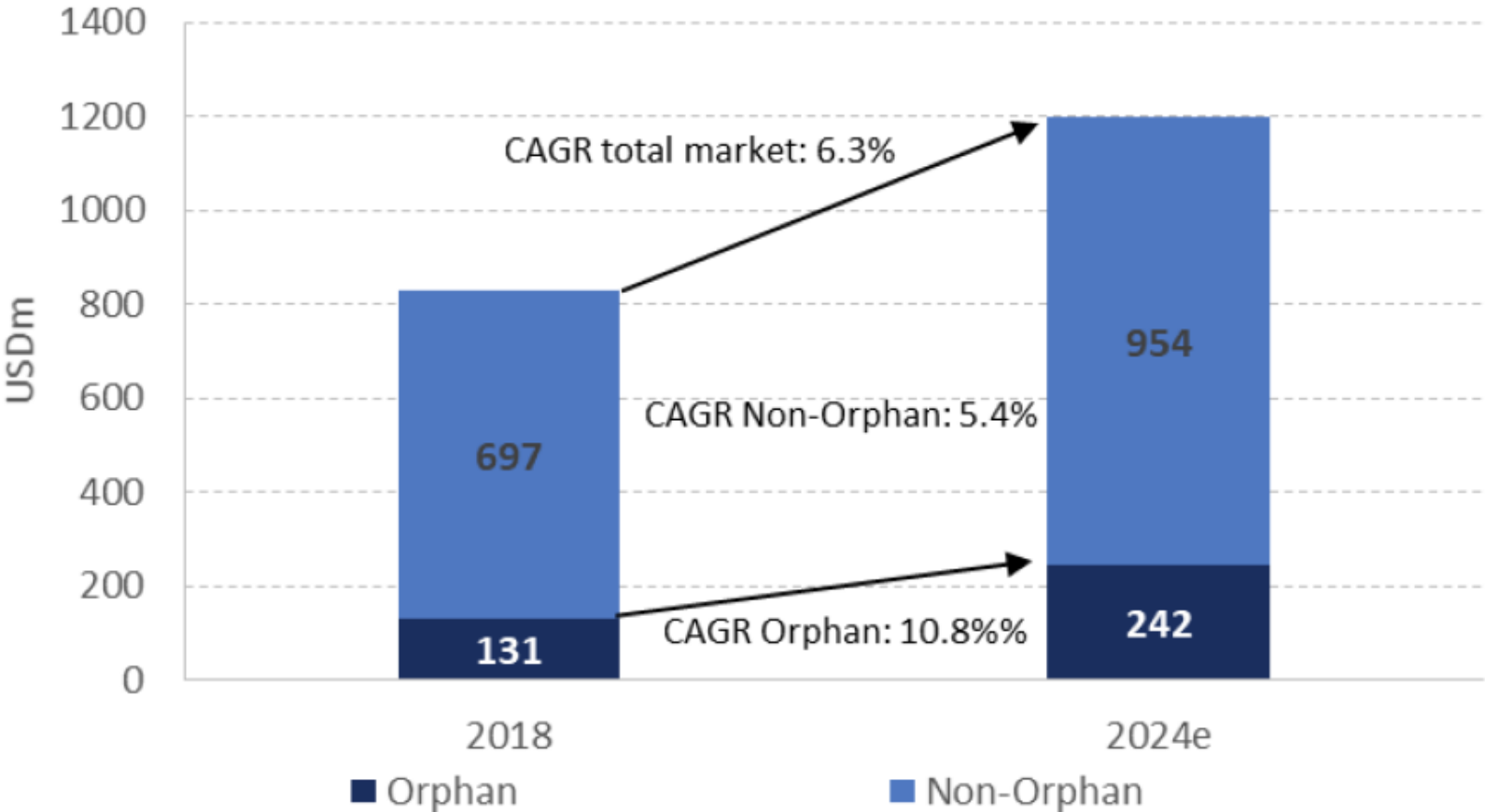
Orphan drug segment – a highly attractive opportunity

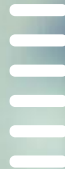


Well-defined patient populations with substantial unmet medical need

CAGR estimates of total pharmaceutical market vs orphan

The global orphan or rare disease market size was valued at an estimated USD 140 – 150 bn and is expected to grow at 10-14% CAGR over the coming five years.





5.

Summary

Commercialisation of *Emcitate* & *Aladote*

Commercial infrastructure build up initiated



Strong success factors...

- 1 High unmet medical need without competing compounds
- 2 Centralized, **focused target** groups of **specialists**
- 3 **Top-down** scientific **sales approach**
- 4 Leading KOL support
- 5 Treatment algorithms **highly protocol driven**



...for sustainable, profitable & lean commercialisation

- Building **inhouse commercial capabilities** for launch of *Emcitate*® and *Aladote*® in EU and US
- **Small and focused footprint** with an estimated < 50 FTEs considered sufficient for both assets
- Retain **larger share of product revenues** over time within Company
- **Commercialisation** in other territories through **partners**

Egetis – a de-risked biotech with substantial unlocked potential



- Late stage biotech “under the radar”, developing the first therapy for a devastating genetic disorder
 - Strong team with established track record in the orphan drug space, including SOBI, Alexion, Biomarin, Biogen, Vertex, Sarepta, Shire and Wilson Therapeutics
- Strong data in clinical trials, demonstrating significant effects on key clinical outcomes
 - Supported by strong mechanistical rationale and data from animal models
- High likelihood to reach first approval in 2024/25, already passed most of typical drug development risks
 - Submitted application for regulatory approval of Emcitate for MCT8 deficiency in EU (MAA) – Submitted October 9, 2023
 - A small trial reconfirming the effect on biomarker T3 under way to complete the US dossier
- Significant market opportunity with potential for premium orphan drug pricing
 - Disease awareness activities already bearing fruits
 - Continuous expansion of the Emcitate Managed Access Program confirms high unmet medical need
- Eligible for priority review voucher upon US approval, which can be sold for ~100-150 MUSD

Two highly promising orphan drug candidates

Emcitate® – Therapy for MCT8 deficiency

- MCT8 deficiency affects ~1:70,000 males: high unmet medical need, no available treatment. No competing sponsored products in clinical development
- Orphan Drug Designation in EU & US
- US Rare Pediatric Disease Designation, eligible for **Priority Review Voucher**. Fast track designation granted by FDA
- Triac Trial I (Phase IIb) completed with **significant** and **clinically** relevant effects on **T3 levels** and **chronic thyrotoxicosis**
- Real-world data published **2021 confirms long-term efficacy and safety** of *Emcitate*
- **MAA** filed in **October 2023**
- **Pivotal ReTRIACt trial for US NDA is ongoing**. A 30 days placebo-controlled trial in at least 16 patients to verify the results on T3
- **Triac Trial II**: Topline results announced June 19, 2024. Did not meet primary endpoints. Confirmed the significant and durable reduction of T3 levels in all patients
- Over **220 patients** are being **treated** with *Emcitate* in managed access programs— Expanded Access Program implemented in the US as requested by the FDA

Aladote® – To prevent acute liver injury caused by paracetamol poisoning*

- Paracetamol poisoning is one of the most common overdoses with >175,000 hospital admissions globally per annum
- No adequate treatment exists for increased risk patients
- Orphan drug designation (ODD) granted in the US & EU
- Successful results from Phase Ib/IIa study in paracetamol overdosed patients
- Pivotal Phase IIb/III study planned for marketing authorization application in both US and EU
- No competing products in clinical development
- In-house development parked until *Emcitate* submissions have been completed for MCT8 deficiency

*In-house development of *Aladote* parked until *Emcitate* submissions have been completed

Pipeline milestones



An integrated orphan drug company, focusing on late-stage development for commercialization



1

Dedicated orphan drug company
Two late-stage assets: ***Emcitate*** and ***Aladote****

2

Emcitate MAA filed in **October 2023**
Pivotal trial for ***Emcitate*** NDA is ongoing

3

Highly attractive **orphan drug segment**

4

Plan to **launch** through **small in-house commercial** organization in the EU and North America

5

Strong team with late-stage orphan clinical development, registration and commercialization experience from:

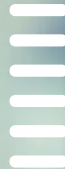


Listed on NASDAQ Stockholm (EGTX)

HQ in Stockholm, Sweden

~40 FTEs

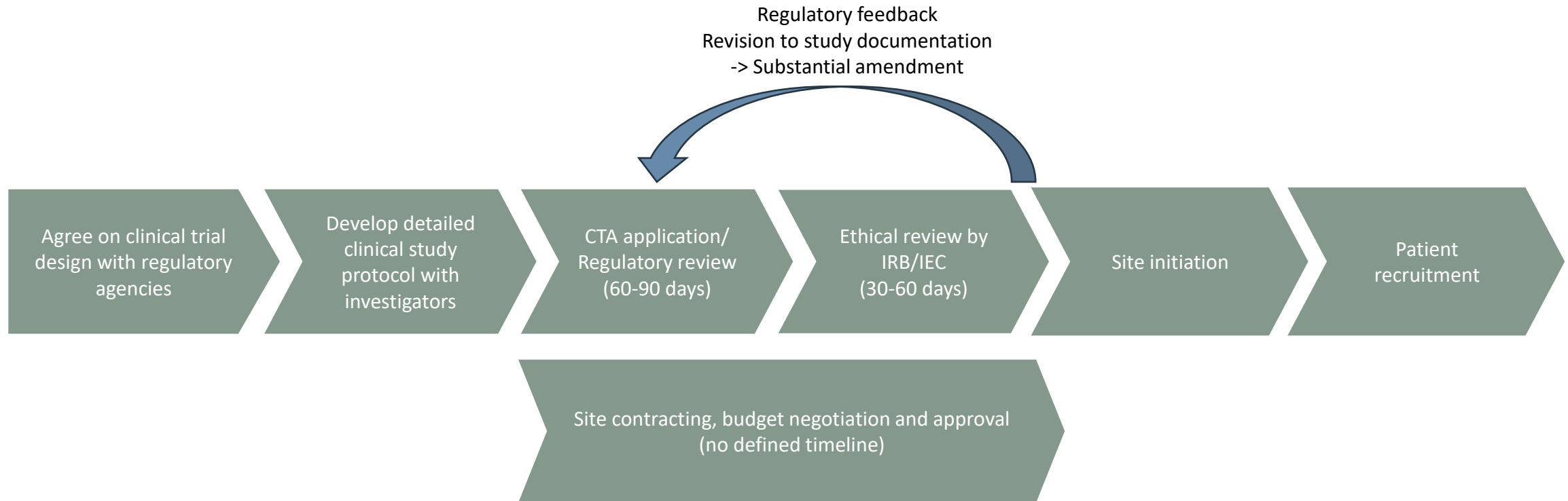
*In-house development of *Aladote* parked until *Emcitate* submissions have been completed



A.

Appendix

Roadmap to site initiations in the ReTRIACt trial



- Longer than anticipated review times for IRB/IEC and site contracting as well as the need for a substantial amendment of study documentation led to significant delays in site initiation.

Termination of discussions regarding a potential acquisition of the Company



Announcement published on May 23, 2023

- Discussions, triggered by an unsolicited approach by an external party, have taken place between certain external parties and Egetis regarding a potential acquisition of the Company
- Discussions have now been terminated as the Board believes the contemplated offer and terms, while providing a premium to the current share price, considerably undervalued the long-term prospects of the Company
- *“A transformative period for the Company, with several near-term value creating milestones and the Board of Egetis believes that the strategy to build an independent sustainable rare-disease company life remains the most long-term value creating alternative for our shareholders”*
- As a consequence of this intense process and discussions, the timeline for the submission of the marketing authorisation application (MAA) for *Emcitate* (tiratricol) to the European Medicines Agency (EMA) has been extended from the second quarter to the early autumn of 2023*

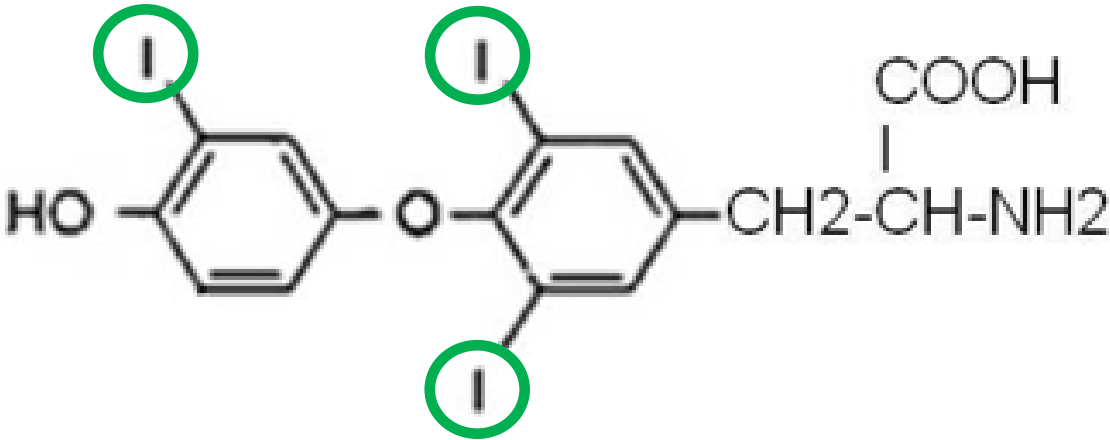
* *Emcitate* MAA filed in October 2023

Discovery of *Emcitate* (Triac, tiratricol)

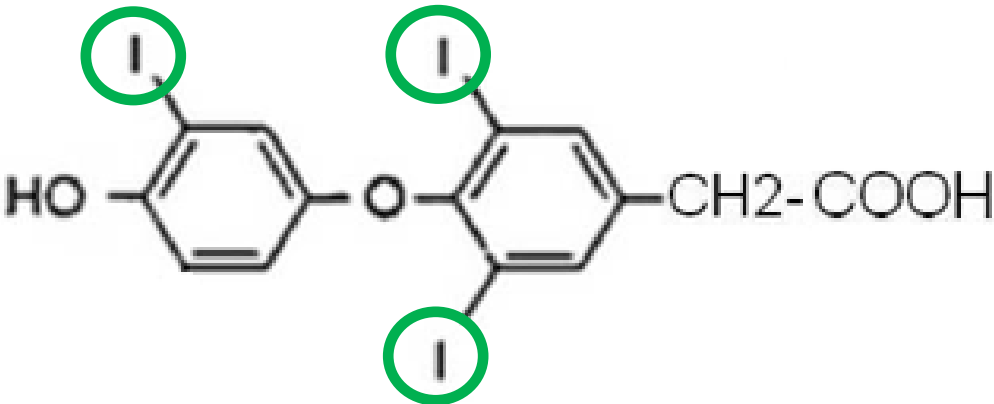


ROSALIND PITT-RIVERS
M.Sc., Ph.D. Lond.

T3



Triac
(tiratricol)



Preliminary Communication

PHYSIOLOGICAL ACTIVITY OF THE
ACETIC-ACID ANALOGUES OF SOME
IODINATED THYRONINES

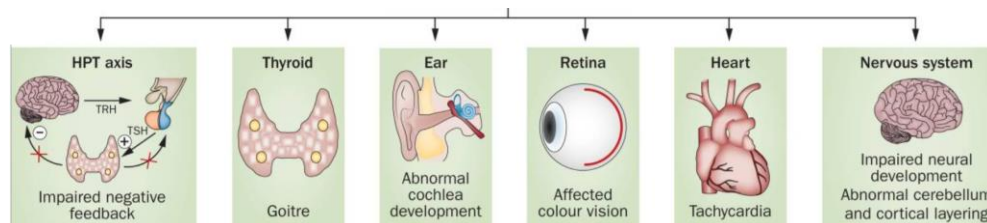
Resistance to Thyroid Hormone type Beta (RTH-β)

Potential indication expansion for Emcitate into non-overlapping patient population

Characteristics of RTH-β

- Caused by mutations in thyroid hormone receptor beta (TRβ)¹
- Reduced target tissue response to thyroid hormone in TRβ dependent tissues
- Incidence 1:20,000 to 1:40,000 (both genders)
- Clinical heterogeneity, ranging from mild to severe
- Diagnosis: High T3&T4, normal/high TSH; confirmed by sequencing of the TRβ gene
- Clinical phenotypes: goiter, CV issues, failure to thrive, neurocognitive dysfunction

Overview of tissues affected in RTH-β



Emcitate as potential treatment for RTH-β

- *Emcitate* efficacious in restoring signaling in majority of TRβ mutations *in vitro*
- Initial clinical experience demonstrates positive effects on key clinical symptoms in RTH-β patients, including cardiovascular, thyrotoxic and neuropsychiatric symptoms²
- Mechanistic rationale: *Emcitate* has a higher affinity than T3 for several TRβ-mutants identified
- *Emcitate* received orphan drug designation for RTH-β from FDA and EMA in 2022
- Development plan for *Emcitate* in RTH-β under evaluation

References:

1. Pappa & Refetoff (2021) Front. Endocrinol. 12, 656551
2. Anzai et al. (2012) Thyroid 22, 1069-1075

Leadership team with global experience & proven track record



Nicklas Westerholm

CEO

- Joined 2017
- AstraZeneca 1995-2017
- VP Late-stage development CVMD
- Executive Officer & VP Japan Operations
- Director Investor Relations



Desiree Luthman

VP Regulatory Affairs

- Joined 2023
- Global regulatory professional, >25y experience
- Passage Bio, Verona Pharma, Sanofi, BMS, Celgene, AstraZeneca



Katayoun Welin-Berger, PhD

VP Technical Operations

- Joined 2023
- VP Operations at Calliditas Therapeutics
- Previously at BioGaia and AstraZeneca



Yilmaz Mahshid, PhD

CFO

- Joined 2021
- Investment Manager & Controller at Industrifonden
- Sell-side analyst at Pareto & Öhman
- CEO Medivir



Kristina Sjöblom Nygren, MD

CMO

- Joined 2020
- CMO, Head of Development at Santhera
- 18 years at SOBI, Wyeth & AstraZeneca
- Worked as physician in clinical positions



Anny Bedard

President Egetis North America

- Joined 2022
- Commercial leadership roles at Shire and Sarepta



Christian Sonesson, PhD

VP Product Strategy & Development

- Joined 2017
- AstraZeneca 13 years
- Late-stage development expertise from FORXIGA, MOVANTIK, ONGLYZA, BRILINTA & QTERN



Henrik Krook, PhD

VP Commercial Operations

- Joined 2020
- Commercial roles at Alexion, Novartis, Roche and Affibody



Nils Hallen

Global Head of HR

- Joined 2021
- Adjunct professor in work & organizational psychology



Laetitia Szaller

General Counsel & Head of Compliance

- Joined 2023
- Senior legal roles at AM Pharma, UCB & Zoetis



Karl Hård, PhD

VP IR & Business Development

- Joined 2022
- Redx Pharma, Optimum Strategic Communications, Kiadis, AstraZeneca

Board of directors



Mats Blom

Chair of the board since 2024

- Shares in Egetis: 3,134,762
- BA, Business Administration & Economics, Lund University; MBA, IESE University of Navarra
- Other assignments: CFO NorthSea Therapeutics, Board member Hansa Biopharma, Auris Medical, Altamira Therapeutics & Pephexia Therapeutics



Thomas Lönngren

Board member since 2021

- Shares in Egetis: 283,158
- MSc in social and regulatory pharmacy and a degree in Pharmacy, University of Uppsala.
- Previously Executive Director of the European Medicines Agency
- Other assignments: Board member Compass Pathways & NDA Group



Gunilla Osswald

Board member since 2017

- Shares in Egetis: 40,000
- PhD in biopharmacy and pharmacokinetics
- Other assignments: CEO BioArctic AB



Elisabeth Svanberg

Board member since 2017

- Shares in Egetis: 37,676
- MD, PhD, Assoc Professor in surgery
- Other assignments: Chief Development Officer Ixaltis SA. Board member Leo Pharma, Amolyt Pharma, Galapagos and EPICS Therapeutics



Behshad Sheldon

Board member since 2023

- Shares in Egetis: 0
- BS in neuroscience
- Other assignments: Chair of the Board of FORCE (Female Opioid Research and Clinical Experts) in Princeton, NJ, Board Member, Camurus AB and Maxona Pharmaceuticals; EVP & MD, Biotech Value Advisors

Share Register, Cash and Market Cap



10 Largest shareholders

Name	Capital	Votes	Num. of shares	Verified
Frazier Life Sciences	13.22%	13.22%	38 675 501	2023-12-31
Cidro Förvaltning AB (Peter Lindell)	10.05%	10.05%	29 406 830	2024-09-26
Cetoros AB (Peder Walberg)	9.40%	9.40%	33 776 221	2024-10-31
Fjärde AP-fonden	7.32%	7.32%	21 404 690	2024-09-26
Avla Holding AB (Kennet Rooth)	6.04%	6.04%	17 668 330	2024-09-26
Unionen	4.00%	4.00%	11 700 000	2024-09-26
RegulaPharm AB (Gudrun Hörnqvist)	3.60%	3.60%	10 531 660	2024-09-26
Handelsbanken Fonder	3.46%	3.46%	10 126 235	2024-09-30
Linc AB (Bengt Julander 65.2%)	2.57%	2.57%	7 532 021	2024-09-30
Flerie Invest AB	2.46%	2.46%	7 205 035	2024-09-26
Total 10	62.12%	62.12%	188 026 523	
Total number of owners	8,243			2024-10-31
Total number of shares	359,238,126			2024-10-31

- **Cash position September 30, 2024:** SEK 130M
- **Directed issue Sep 30, 2024:** SEK 282M (net)
- **Number of outstanding shares:** 359,238,126
- **Market Cap:** ~SEK 1.9 billion*
- **Listing venue:** Nasdaq Stockholm, Main Market
- **Ticker:** EGTX

Source: Monitor by Modular Finance. Compiled and processed data from various sources, including Euroclear, Morningstar and the Swedish Financial Supervisory Authority (Finansinspektionen). The verification date may vary for certain shareholders

* Nov 15, 2024

Acquisition of Rare Thyroid Therapeutics on November 5, 2020

The combination will drive synergies

PledPharma and Rare Thyroid Therapeutics merged to launch a new company



PledPharma

- Team with profound late-stage drug development experience and strong track-record
- Listing on Nasdaq Stockholm provides access to public markets and capital as well as visibility
- Desired prospective partner in project collaborations. Previous major license agreement with Solasia
- Efficient internal organisation and strong corporate governance

Rare Thyroid Therapeutics

- Team with strong track-record of identifying and developing ODDs and creating shareholder value
- Strong network of external project advisors with specialist knowledge. Collaboration with Erasmus Medical Center in Rotterdam
- Founding team with experience from international launch and commercialisation of orphan drugs

Synergistic orphan drug focus

- 2020 accelerated PledPharma's strategic review
- Lead asset Aladote® facilitates the new pronounced strategic focus on orphan drug segment
- Emcitate® and RTT's capabilities fit well with the new strategy
- Build critical mass, generate synergies and improve operational effectiveness for projects in the orphan segment
- Size, vicinity and complementary capabilities allow for a fast and smooth integration

The acquisition and rights issue

Institutional investor base broadened



Acquisition

- ✓ On 5 November 2020, PledPharma acquired all outstanding common shares in Rare Thyroid Therapeutics
- ✓ Consideration consisted of a combination of PledPharma common shares and cash
 - An upfront cash payment of SEK 60m
 - 63.8 million shares representing approx 39% of the total number of outstanding shares in PledPharma post rights issues
 - Owners of Rare Thyroid Therapeutics will receive a royalty of 3% of net sales generated through Emcitate®¹
 - Owners of Rare Thyroid Therapeutics will also be granted 50% of the net proceeds from a potential sale of US Rare Pediatric Disease Priority Review Voucher related to Emcitate®

Rights issue

- ✓ Successfully raised SEK 250 million in oversubscribed rights issue (c. SEK 200m) and utilized overallotment option (c. SEK 50m)
 - Subscription price of SEK 5.25 per share corresponding to a 2.5 percent premium to close 2 October 2020
- ✓ Institutional investor base broadened
 - Overallotment Option, allocated to the Fourth Swedish National Pension Fund (“AP4”), NYIP (Nyenburgh Holding BV) and Nordic Cross
 - The proceeds will be used to finance: (i) the development of Emcitate® and Aladote® to market approval in Europe and USA (60%); (ii) initial commercial preparations (20%); (iii) general corporate purposes and financial flexibility (20%)

Thank you!

Egetis Therapeutics
egetis.com
karl.hard@egetis.com