EGETIS THERAPEUTICS

WE CARE

FOR THE RARE

Corporate presentation

October 2023

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

Disclaimer

THIS PRESENTATION AND ITS CONTENTS ARE NOT FOR RELEASE, PUBLICATION OR DISTRIBUTION, IN WHOLE OR IN PART, DIRECTLY OR INDIRECTLY, IN OR INTO OR FROM THE UNITED STATES OF AMERICA, CANADA, AUSTRALIA, JAPAN OR ANY JURISDICTION WHERE SUCH DISTRIBUTION IS UNLAWFUL.

IMPORTANT: You must read the following before continuing. The following applies to this document, the oral presentation of the information in this document by Egetis Therapeutics AB (the "Company") or any person on behalf of the Company, and any question-and-answer session that follows the oral presentation (collectively, the "Information"). In accessing the Information, you agree to be bound by the following terms and conditions.

The Information is not intended for release, publication or distribution, in whole or in part, directly or indirectly, in or into or from the United States of America, Canada, Australia, Japan or any other jurisdiction where such distribution would be unlawful. This presentation is not a prospectus or similar document and it has not been approved, registered or reviewed by the Swedish Financial Supervisory Authority nor any governmental authority or stock exchange in any jurisdiction.

The Information has been prepared by the Company and is intended to present background information on the Company, its business and the industry in which it operates. The Information contains summary information only and does not purport to be comprehensive and is not intended to be (and should not be used as) the sole basis of any analysis or other evaluation. The Information does not constitute or form part of and should not be construed as an offer or the solicitation of an offer to subscribe for or purchase securities of the Company, and nothing contained therein shall form the basis of or be relied on in connection with any investment activity. The Company shall not have any liability whatsoever (in negligence or otherwise) for any loss whatsoever arising from any use of the Information or otherwise arising in connection with this presentation.

By accessing this Information, you represent that such access does not violate any registration requirements or other legal restrictions in the jurisdiction in which you reside or conduct business. It is especially noted that the Information may not be accessed by persons within the United States or "U.S. Persons" (as defined in Regulation S under the Securities Act of 1933, as amended (the "Securities Act") unless they are qualified institutional buyers "QIBs" as defined in Rule 144A under the Securities Act. By accessing the Information, you represent that you are (i): a non-U.S. person that is outside the United States or (ii) a QIB. Further, the Information may not be accessed by persons within the United Kingdom unless they are persons with professional experience in matters relating to investments falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 as amended (the "Order"), a "qualified investors" falling within Article 2(e) of Regulation (EU) 2017/1129 as it forms part of English law by virtue of the European Union (Withdrawal) Act 2018 ("EUWA"), or high net worth entities falling within Article 49(2)(a) to (d) of the Order (each a "Relevant Person"). By accessing the Information, you represent that you are: (i) outside the United Kingdom or (ii) a Relevant Person.

The Information contains forward-looking statements. All statements other than statements of historical fact included in the Information are forward-looking statements. Forward-looking statements give the Company's current expectations and projections relating to its financial condition, results of operations, plans, objectives, future performance and business. These statements may include, without limitation, any statements preceded by, followed by or including words such as "target," "believe," "expect," "aim," "intend," "may," "anticipate," "estimate," "plan," "project," "will," "can have," "likely," "should," "would," "could" and other words and terms of similar meaning or the negative thereof. Such forward-looking statements involve known and unknown risks, uncertainties and other important factors beyond the Company's control that could cause the Company's actual results, performance or achievements to be materially different from the expected results, performance or achievements expressed or implied by such forward-looking statements. Such forward-looking statements are based on numerous assumptions regarding the Company's present and future business strategies and the environment in which it will operate in the future. As a result, you are cautioned not to place undue reliance on such forward-looking statements.

No representation, warranty or undertaking, express or implied, is made by or on behalf of the Company as to, and no reliance should be placed on, the fairness, accuracy, completeness or correctness of the Information or the opinions contained therein. The Information has not been independently verified and will not be updated. The Information, including but not limited to forward-looking statements, applies only as of the date of this document and is not intended to give any assurances as to future results. The Company expressly disclaim any obligation or undertaking to disseminate any updates or revisions to the Information, including any financial data or forward-looking statements, and will not publicly release any revisions it may make to the Information that may result from any change in the Company's expectations, any change in events, conditions or circumstances on which these forward-looking statements are based, or other events or circumstances arising after the date of this document. Market data used in the Information not attributed to a specific source are estimates of the Company and have not been independently verified.

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* submissions have been completed

WE CARE

FOR THE RARE

1.

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

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**
   Target *Emcitate* **NDA 2024**
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

~30 FTEs

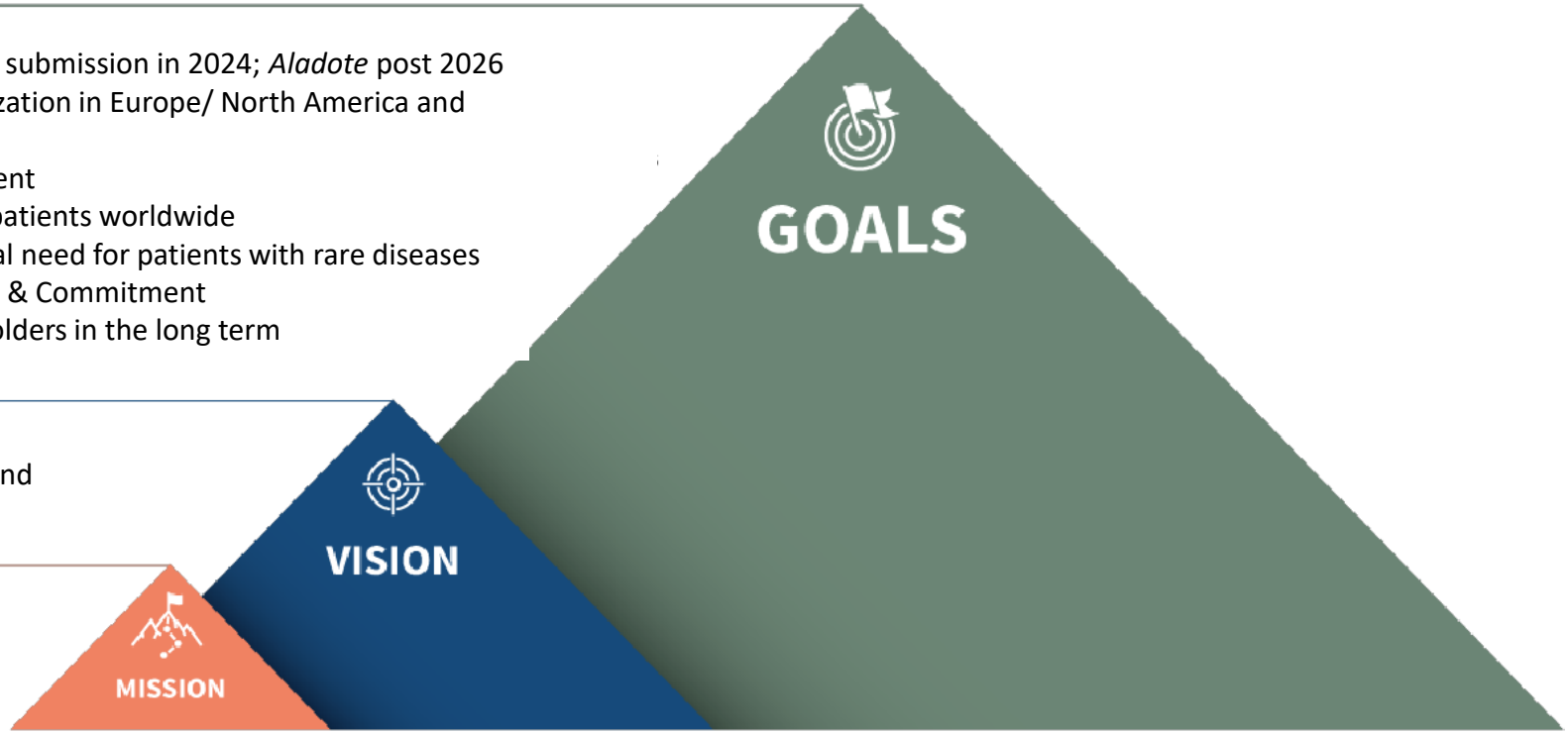
*In-house development of *Aladote* parked until *Emcitate* submissions have been completed

Building a sustainable orphan drug company

WE CARE

FOR THE RARE

- Successfully develop *Emcitate* for EU submission in 2023 and US submission in 2024; *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 extend and improve quality of 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

Termination of discussions regarding a potential acquisition of the Company

Announcement published on May 23, 2023

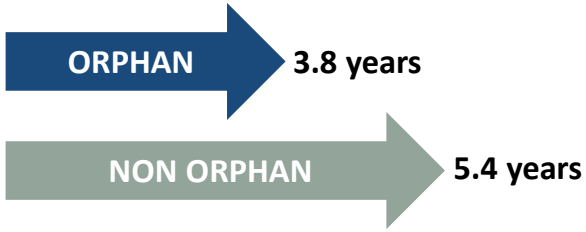
- Discussions, triggered by an unsolicited approach by an external party, have taken place between certain external parties and Egetis regarding a potential acquisition of the Company
- Discussions have now been terminated as the Board believes the contemplated offer and terms, while providing a premium 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

Orphan drug segment – a highly attractive opportunity

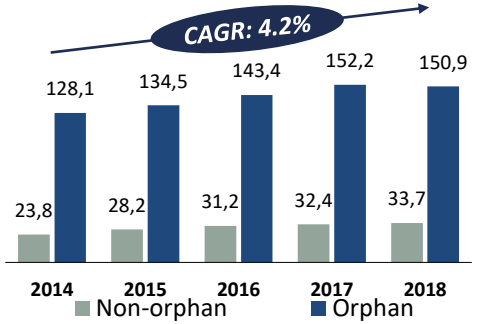
Shorter clinical development time[1]

Phase II to launch Average # of years



Higher attainable prices[2]

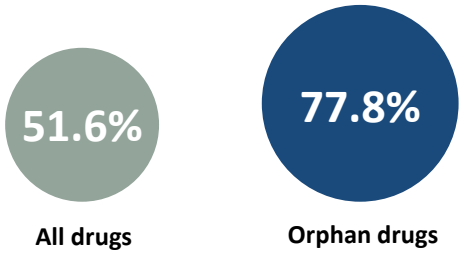
Mean cost per patient and year (USDk)



Higher probability of success[3]

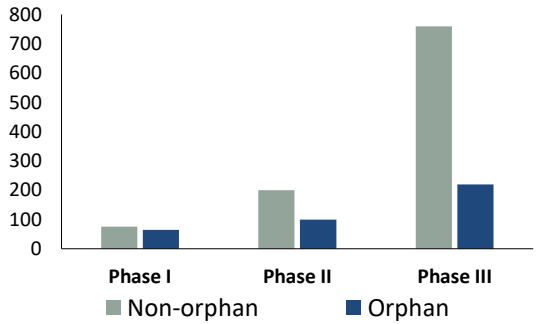
Phase III to approval

POS in metabolic/endocrinology indications

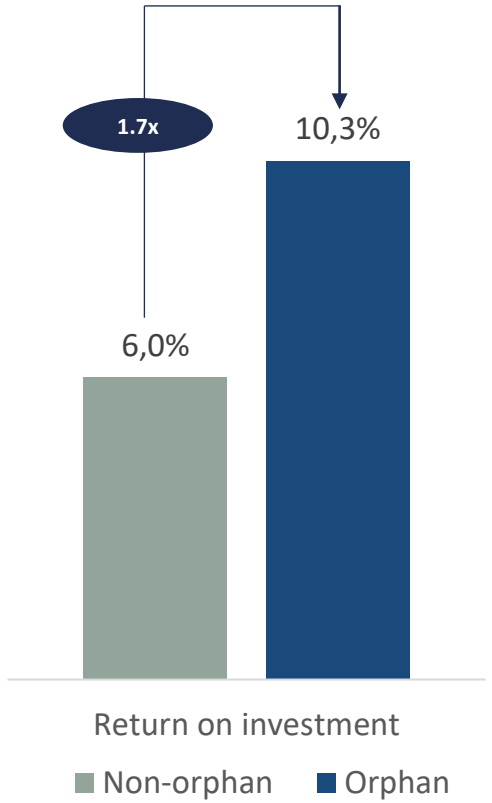


Fewer patients for clinical trials[4]

Patients per trial



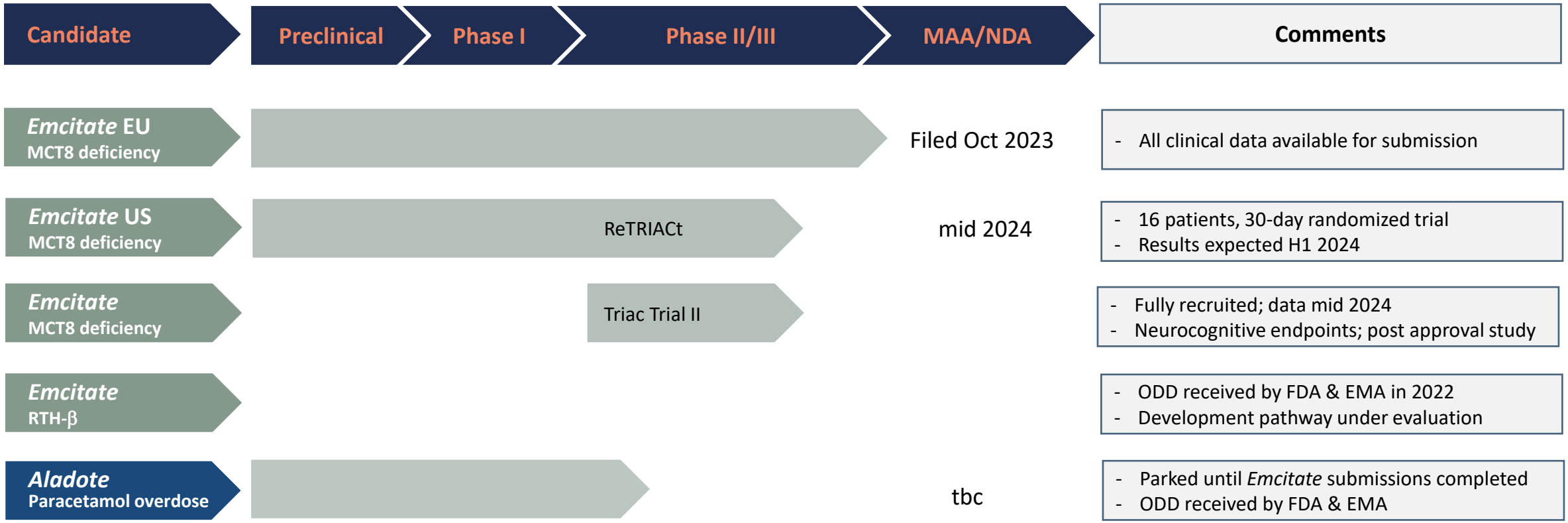
Orphan drugs attractive returns[5]



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

Pipeline overview

Planned Emcitate EU filing in 2023 and US filing in 2024



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,** based on existing clinical data
- **NDA** in **mid 2024** under fast-track designation, after conducting a 30 days placebo-controlled study (ReTRIACt) in 16 patients to verify the results on T3
- **Triac Trial II fully recruited**; to establish the effects of early intervention on **neurocognitive** development, previously seen in Triac Trial I. Results expected mid 2024
- Over **180 patients** are being **treated** with *Emcitate* on a **named patient basis** – Expanded Access Program implemented 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

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**

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 2002 the first thyroid hormone transporter (MCT8) was identified
  - 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

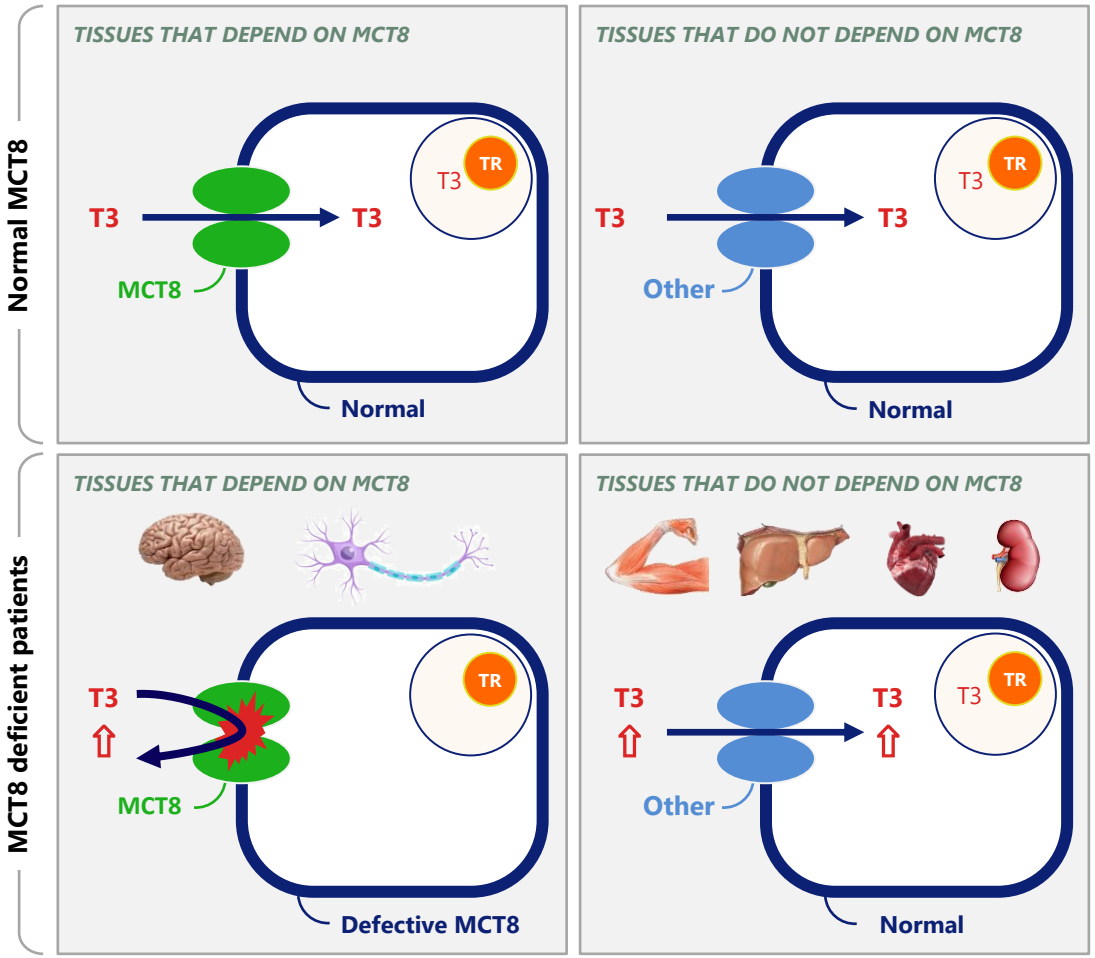
MCT8 Plays a Key Role in Neurocognitive Development

- 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?

- Genetic X-linked disorder
- Impaired thyroid hormone trafficking across cellular membranes
- MCT8 is a key thyroid hormone transporter in the body
- Prevalence 1:70,000 males

Patients with MCT8 Deficiency[1)]

What does it mean?

- Non-functional MCT8 protein: T3 cannot cross blood-brain-barrier
- Low amounts of thyroid hormone in the brain & CNS
- Disrupted feedback loop results in a compensatory increase in circulating thyroid hormone

- Simultaneous too high & too low thyroid hormone in different tissues

What are the challenges?

- Patients appear normal at birth
- Initial symptoms within the first months of life
- Severe intellectual disability
- Most patients never able to sit or walk; limited ability to communicate
- Life-long morbidity: agitation, CV symptoms, wasting & impaired life expectancy

- Heavily dependent on caregivers resulting in very high disease burden

How do you manage the disease?

- No available therapy
- Easy diagnosis once considered with readily available, low-cost lab-test
- Large proportion of patients remain undiagnosed with significant delay to diagnosis

- Significant unmet medical need: humanitarian, health economic, societal

Quick facts from natural history[2]

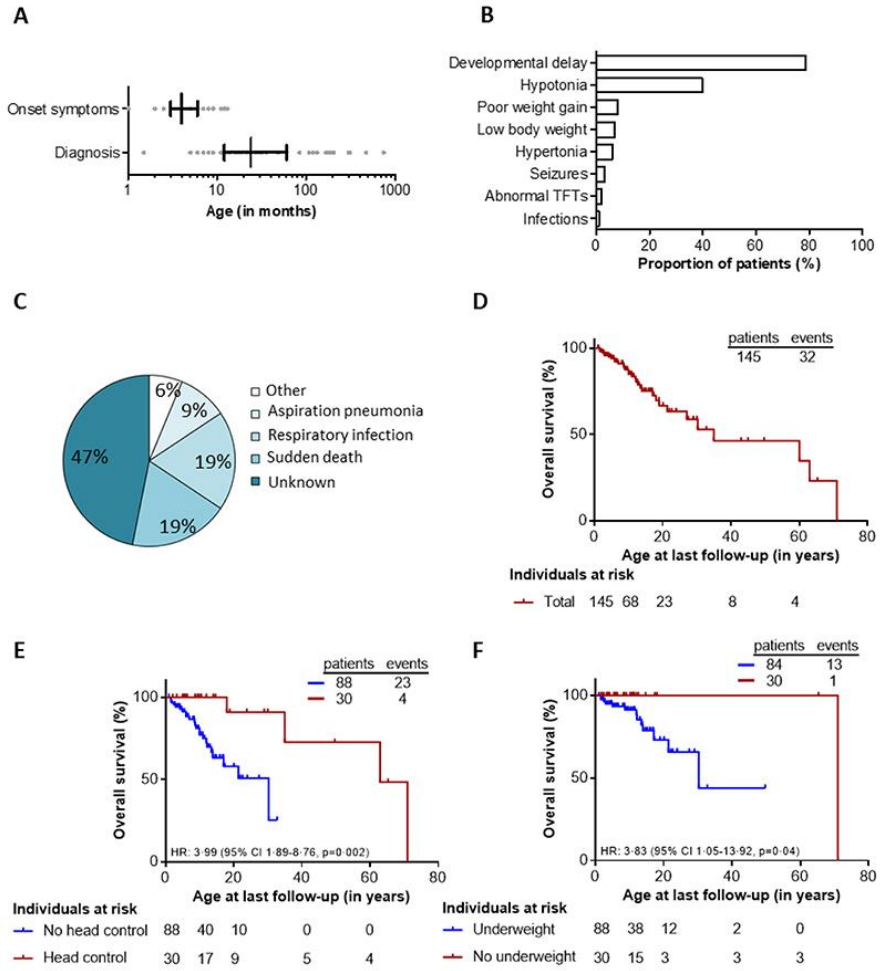
Median onset of symptoms:	**4 months**
Median age of diagnosis:	**24 months**
Patients surviving into adulthood:	**70%**
Severe intellectual disability:	**100%**
Ability to sit independently:	**8%**
Hypotonia, hypertonia & persistence of primitive reflexes:	**90%**
Severe underweight:	**75%**
Cardiac arrythmias (PAC):	**76%**
Median life expectancy:	**35 years**
Life long 24-hour care:	**100%**

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

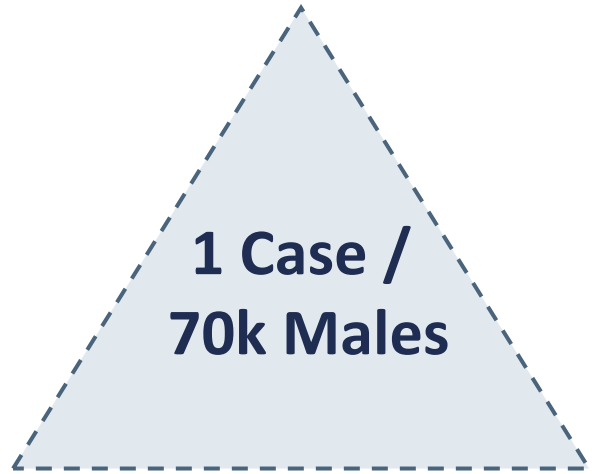
- 151 patients were enrolled with 73 different MCT8 (SLC16A2) mutations
- Median age at diagnosis was 24.0 months
- 21% patients died; the main causes of mortality were pulmonary infection (six patients or 19%) and sudden death (six patients or 19%)
- Median OS was 35.0 years (95% CI 8.3-61.7)
- Individuals 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

Relevant Sources & Data	Available Data Leads to Consistent MCT8 Deficiency Incidence Estimates
Visser et al., Clinical Endocrinology 2013	• Multiple cohorts of patients with X-linked mental retardation under study • MCT8 deficiency prevalence in studied populations implies a 1:50k-100k Male incidence perimeter
Neonatal Screening - Netherlands	• 140k births & 70k Males a year with 1-2 diagnosed cases a year on average over the past years • Implies more than 1:70k incidence
Triac Trial II - Germany	• 20 months of screening and 400k live births yielded 12 patients below 30 months of age • Implies at least ~1:30k incidence

Supporting our Conservative Estimate



2.

Clinical experience with Emcitate

Orphan drug candidate

with clear scientific and mechanistic rationale and established safety profile

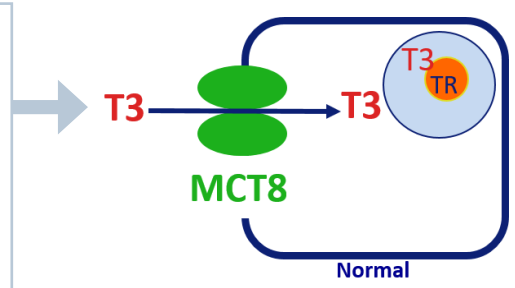
Difference normal MCT8 and deficiency of MCT8

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

Normal MCT8 ✔

- Functional thyroid gland producing T3
- Production of functional MCT8

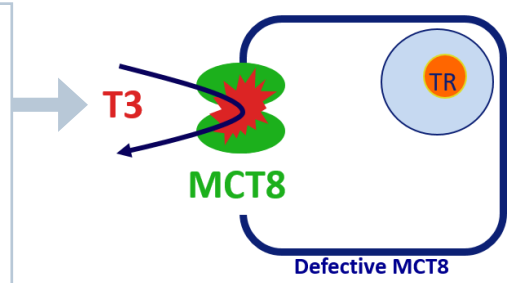
→ T3 cross cell membrane and enters target cell



Mutated MCT8 ✘

- Functional thyroid gland producing T3
- Absence or loss of function of MCT8 on the cell surface

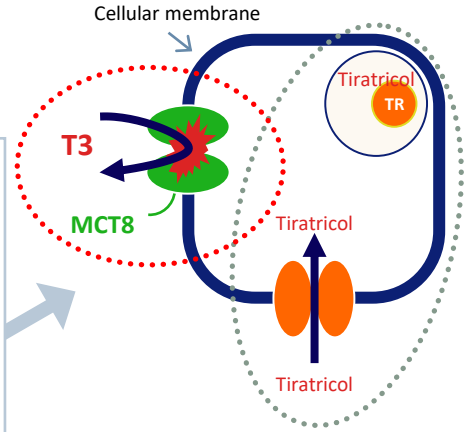
→ T3 cannot cross cell membrane and fails to enter cells



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**, early intervention trial in young subjects to establish the effect on neurocognitive development, previously seen in Triac Trial I. Fully recruited Q2 2022, 22 patients. Results expected mid 2024

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, based on existing clinical data**
- US **NDA submission planned mid 2024**: A 30-day, placebo-controlled study in 16 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 **180 patients** are being treated with *Emcitate* on an individual license or compassionate use basis, 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

- Evaluate the efficacy and safety of oral administration of tiratricol in male patients with MCT8 deficiency of all ages
- Highly significant primary outcome - Change in T3 serum concentrations
- Safe and tolerable
- Results published in *The Lancet* 2019

Secondary objective and results

- Change in other thyroid hormone function tests, thyrotoxic symptoms and markers
- Significant and clinically relevant effects observed across secondary endpoints

Description

- An international, single-arm, open-label, Phase II trial
- ClinicalTrials.gov identifier: NCT02060474

# of patients

- 46 MCT8 patients in 9 countries

Timetable

- Initiated in 2014 (first patient in)
- Completed in 2018

THE LANCET Articles

Effectiveness and safety of the tri-iodothyronine analogue Triac in children and adults with MCT8 deficiency: an international, single-arm, open-label, phase 2 trial

Stefan Groeneweg, Robin P Peeters, Carla Moran, Athanasia Stoupa, Françoise Auriol, Davide Tonduti, Alice Dicu, Laura Paone, Klara Rozenkova, Jana Malikova, Adri van der Walt, Irenaeus F M de Coo, Anne McGowan, Greta Lyons, Femke K Aarsen, Diana Barca, Ingrid M van Beynum, Marieke M van der Knoop, Jurgen Jansen, Martien Manshande*, Roelineke J Lunsing, Stan Nowak, Carsiaan A den Uil, M Carola Zillikens, Frank E Visser, Paul Vrijmoeth, Marie Claire Y de Wit, Nicole I Wolf, Angelique Zandstra, Gautam Ambegaonkar, Yogen Singh, Yolanda B de Rijke, Marco Medici, Enrico S Bertini, Sylvia Depoorter, Jan Lebl, Marco Cappa, Linda De Meirleir*, Heiko Krude, Dana Craiu, Federica Zibordi, Isabelle Oliver Petit, Michel Polak, Krishna Chatterjee, Theo J Visser*, W Edward Visser

Summary

Background Deficiency of the thyroid hormone transporter monocarboxylate transporter 8 (MCT8) causes severe intellectual and motor disability and high serum tri-iodothyronine (T_3) concentrations (Allan–Herndon–Dudley syndrome). This chronic thyrotoxicosis leads to progressive deterioration in bodyweight, tachycardia, and muscle wasting, predisposing affected individuals to substantial morbidity and mortality. Treatment that safely alleviates peripheral thyrotoxicosis and reverses cerebral hypothyroidism is not yet available. We aimed to investigate the effects of treatment with the T_3 analogue Triac (3,3',5-tri-iodothyroacetic acid, or tiratricol), in patients with MCT8 deficiency.

Methods In this investigator-initiated, multicentre, open-label, single-arm, phase 2, pragmatic trial, we investigated the effectiveness and safety of oral Triac in male paediatric and adult patients with MCT8 deficiency in eight countries in Europe and one site in South Africa. Triac was administered in a predefined escalating dose schedule—after the initial dose of once-daily 350 µg Triac, the daily dose was increased progressively in 350 µg increments, with the goal of attaining serum total T_3 concentrations within the target range of 1·4–2·5 nmol/L. We assessed changes in several clinical and biochemical signs of hyperthyroidism between baseline and 12 months of treatment. The prespecified primary endpoint was the change in serum T_3 concentrations from baseline to month 12. The co-primary endpoints were changes in concentrations of serum thyroid-stimulating hormone (TSH), free and total thyroxine (T_4), and total reverse T_3 from baseline to month 12. These analyses were done in patients who received at least one dose of Triac and had at least one post-baseline evaluation of serum throid function. This trial is registered with ClinicalTrials.gov, number NCT02060474.

Findings Between Oct 15, 2014, and June 1, 2017, we screened 50 patients, all of whom were eligible. Of these patients, four (8%) patients decided not to participate because of travel commitments. 46 (92%) patients were therefore enrolled in the trial to receive Triac (median age 7·1 years [range 0·8–66·8]) . 45 (98%) participants received Triac and had at least one follow-up measurement of thyroid function and thus were included in the analyses of the primary endpoints. Of these 45 patients, five did not complete the trial (two patients withdrew [travel burden, severe pre-existing comorbidity], one was lost to follow-up, one developed of Graves disease, and one died of sepsis). Patients required a mean dose of 38·3 µg/kg of bodyweight (range 6·4–84·3) to attain T_3 concentrations within the target range. Serum T_3 concentration decreased from 4·97 nmol/L (SD 1·55) at baseline to 1·82 nmol/L (0·69) at month 12 (mean decrease 3·15 nmol/L, 95% CI 2·68–3·62; p<0·0001), while serum TSH concentrations decreased from 2·91 mU/L (SD 1·68) to 1·02 mU/L (1·14; mean decrease 1·89 mU/L, 1·39–2·39; p<0·0001) and serum free T_4 concentrations decreased from 9·5 pmol/L (SD 2·5) to 3·4 (1·6; mean decrease 6·1 pmol/L (5·4–6·8; p<0·0001). Additionally, serum total T_3 concentrations decreased by 31·6 nmol/L (28·0–35·2; p<0·0001) and reverse T_3 by 0·08 nmol/L (0·05–0·10; p<0·0001). Seven treatment-related adverse events (transiently increased perspiration or irritability) occurred in six (13%) patients. 26 serious adverse events that were considered unrelated to treatment occurred in 18 (39%) patients (mostly hospital admissions because of infections). One patient died from pulmonary sepsis leading to multi-organ failure, which was unrelated to Triac treatment.

Interpretation Key features of peripheral thyrotoxicosis were alleviated in paediatric and adult patients with MCT8 deficiency who were treated with Triac. Triac seems a reasonable treatment strategy to ameliorate the consequences of untreated peripheral thyrotoxicosis in patients with MCT8 deficiency.

Funding Dutch Scientific Organization, Sherman Foundation, NeMO Foundation, Wellcome Trust, UK National Institute for Health Research Cambridge Biomedical Centre, Toulouse University Hospital, and Una Vita Rara ONLUS.

Lancet Diabetes Endocrinol 2019 Published Online July 31, 2019 http://dx.doi.org/10.1016/S2213-8587(19)30155-X

See Online/Comment http://dx.doi.org/10.1016/S2213-8587(19)30217-7

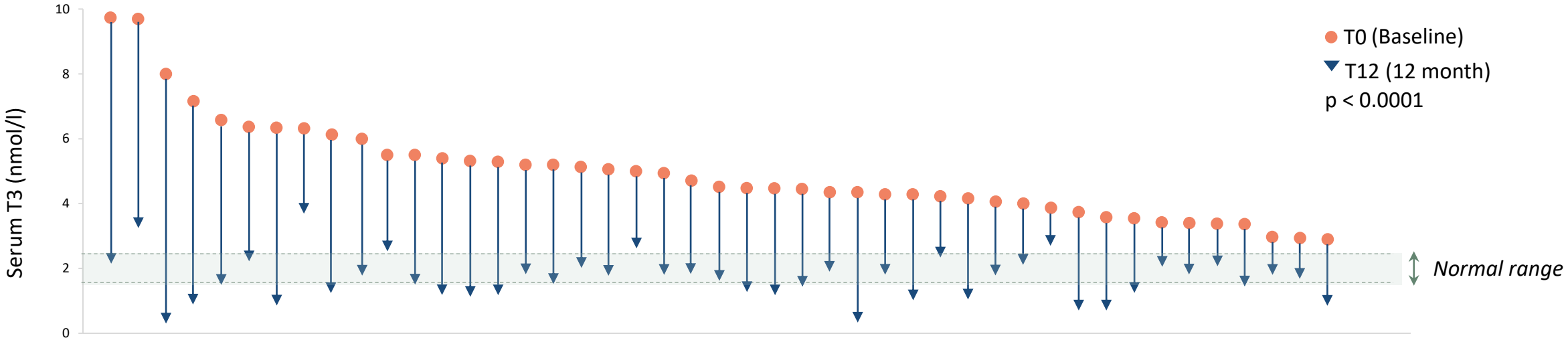
*Dr Manshande died in August, 2018; Prof De Meirleir died in October, 2018; and Prof T J Visser died in March, 2018

Academic Center for Thyroid Diseases (S Groeneweg MD, Prof R P Peeters MD, M Medici MD, Prof T J Visser PhD, W E Visser MD), Sophia Children's Hospital, Division of Paediatric Cardiology (I M van Beynum MD), Sophia Children's Hospital, Department of Paediatric Neurology (I F M de Coo MD, F K Aarsen PhD, M M van der Knoop MSc, M C Y de Wit MD), Department of Cardiology and Intensive Care Medicine (C A den Uil MD), Department of Clinical Chemistry (Prof Y B de Rijke PhD), and Department of Internal Medicine (Prof M C Zillikens MD), Erasmus Medical Centre, Rotterdam, Netherlands; Wellcome Trust-Medical Research Council Institute of Metabolic Science, University of Cambridge, Cambridge, UK (C Moran MB, A McGowan MD, G Lyons RCN, K Chatterjee FRCP); Paediatric Endocrinology, Diabetology and Gynaecology Department, Necker Children's University Hospital, Imagine Institute, Paris, France (A Stoupa M D, Prof M Polak M D); Department of Paediatric Endocrinology and Genetics, Children's Hospital, Toulouse University Hospital, Toulouse, France

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

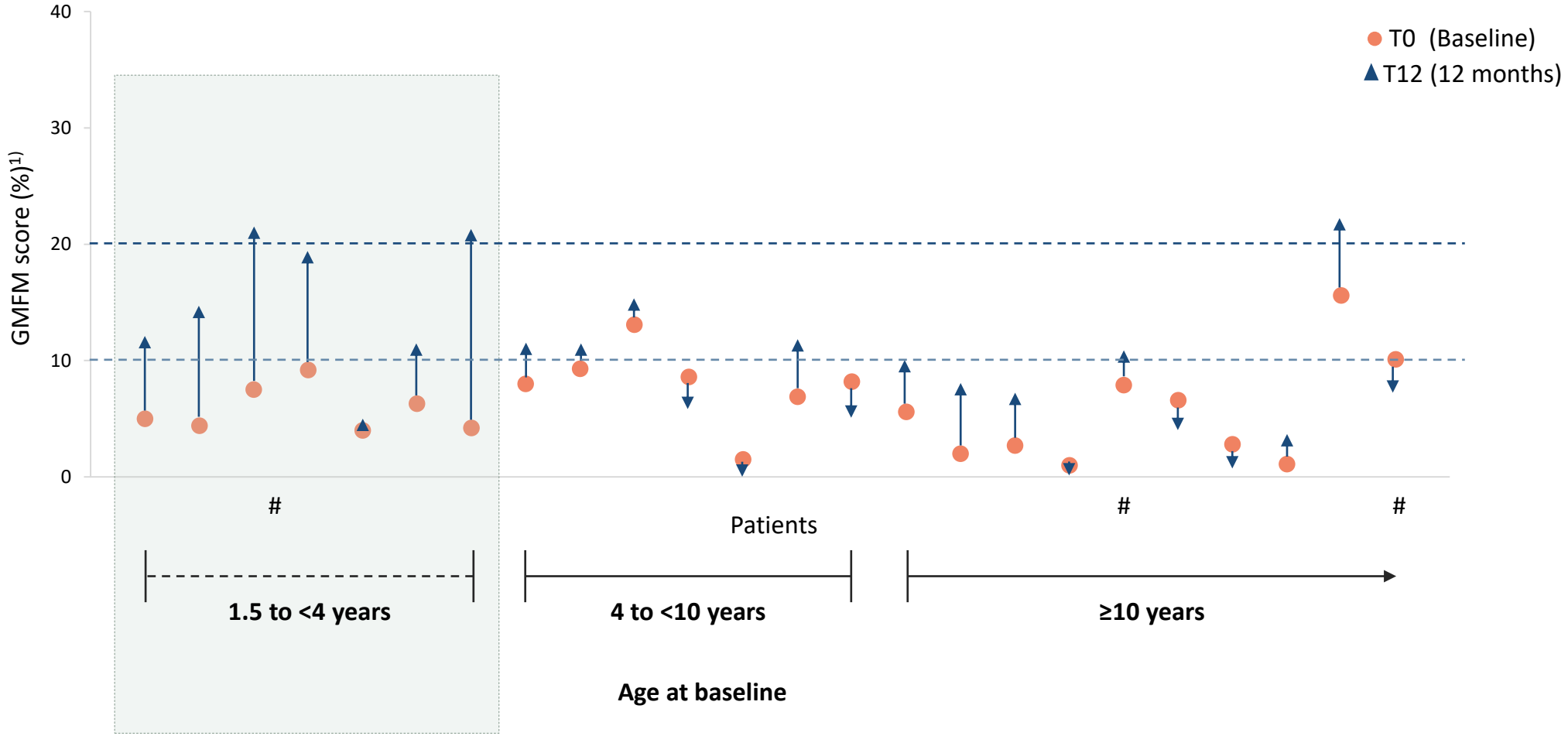


Endpoints	Baseline mean *(± SD)*	12 months mean *(± SD)*	Difference in means *(95% CI)*	p-value
Serum T3 (nmol/L)	4.97 (± 1.55)	1.82 (± 0.69)	-3.15 (-3.62, -2.68)	<0.0001
Weight to age (z score)	-2.98 *(± 1.93)*	-2.71 *(± 1.79)*	0.27 *(0.03, 0.50)*	0.025
Resting heart rate (bpm)	112 *(± 23)*	104 *(± 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

In the youngest patients which is further studied in ongoing, fully recruited, Triac Trial II



Source: Groeneweg et al; Lancet D&E 2019

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

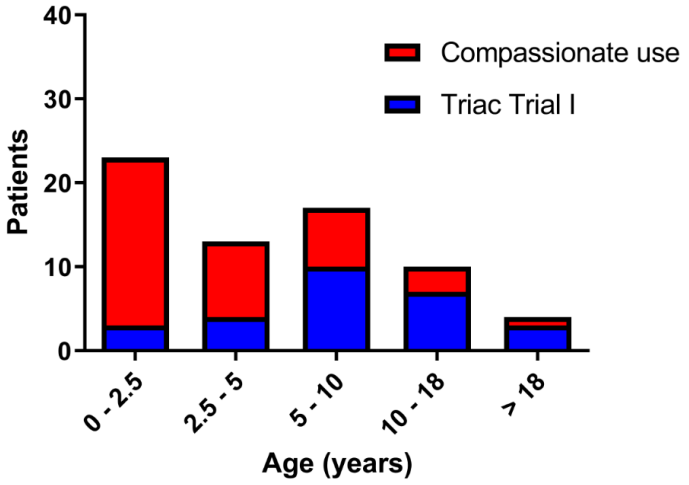
Long term follow up, up to >6 years

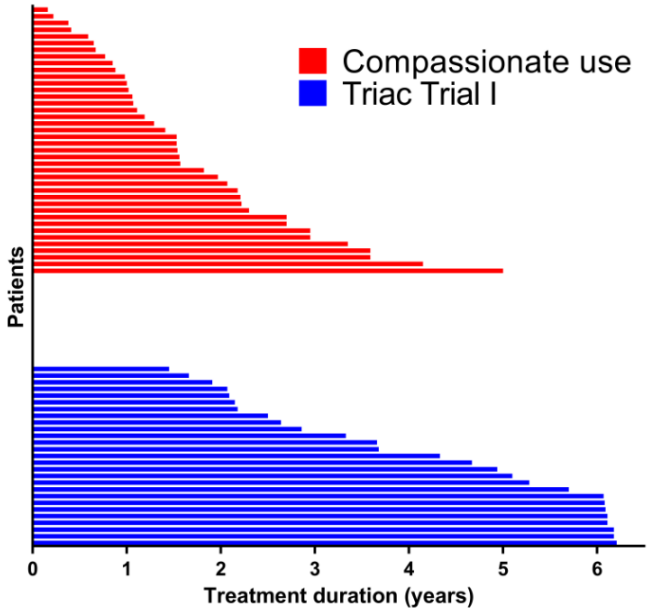
Triac Trial I
N=46

Triac Trial I patients
Available for long-term monitoring
N=27

New patients
From named patient program
N=40

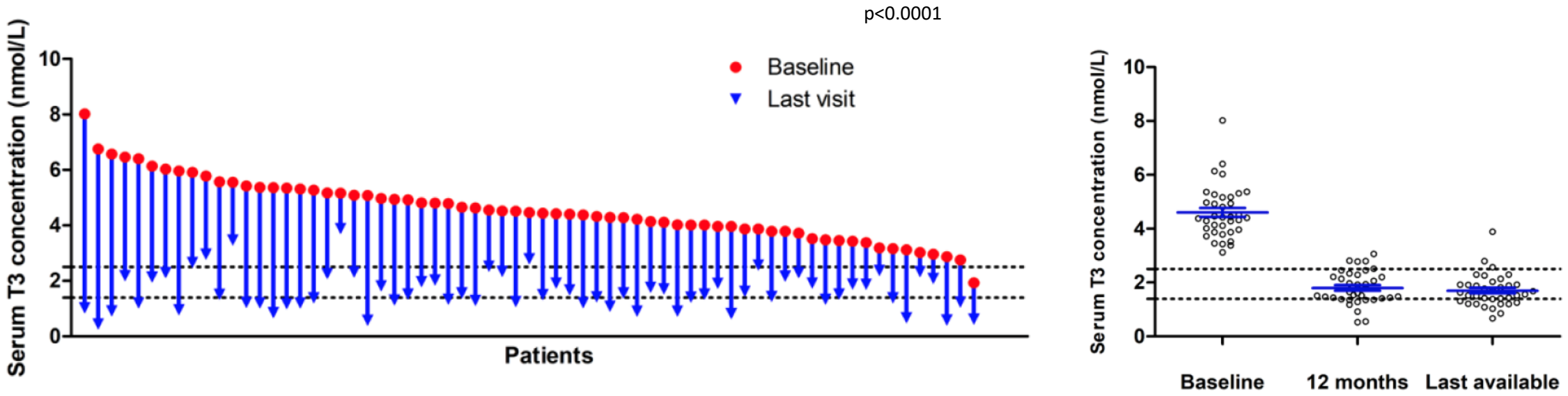
EMC cohort
Published in JCEM
N=67





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				
Anthropometric parameters and heart rate				
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
Thyroid function tests				
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
Peripheral markers				
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)
Eligibility: Six months review of NDA & rolling submission

PRV

Rare pediatric disease designation (FDA)
Eligibility: Priority review voucher upon approval*

MAA
NDA

MAA: Submitted in October ’23
NDA: Small confirmatory study agreed with FDA (submission mid ’24)

ODD

Orphan drug designation for RTH-beta
Eligibility: Market exclusivity for distinct indication

*The voucher may be sold to another sponsor (2021-23 range: ~$100m-$110m)

Emcitate regulatory pathway to submissions 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

Included in MAA in EU submitted on October 9, 2023

Included in NDA in US mid 2024 under the Fast Track Designation

Not required for MAA or NDA submissions

Triac Trial I

- Completed 2018 (Groeneweg, 2019)
- Open-label, international, multi-centre study
- N= 46

EMC cohort study

- New data 2021 (van Geest, 2022)
- N= 27 from Triac Trial I & N= 40 new pts from compassionate use

Natural history

- Retrospective data, 2003 to 2019 (Groeneweg, 2020)
- N= 151

ReTRIACt Trial (Controlled study)

- N= 16
- Placebo controlled
- Top line results H1 2024

Triac Trial II

- Open-label, international, multi-centre study
- Pts ≤ 30 months of age
- Focus on neurocognition
- N= 22
- Full 96 weeks data, expected mid 2024

Data already available

Egetis submitted MAA for Emcitate® to EMA on October 9, 2023, based on existing clinical data

- Based on regulatory interactions, Egetis concludes that **available data** from Triac Trial I and recently published long-term data are **sufficient for a Marketing Authorisation Application (MAA) in Europe**
- Having all clinical data required for regulatory submission already at hand **significantly reduces the remaining risk** for Emcitate
- The ongoing Triac Trial II will continue to further establish the effects of early intervention on the neurocognitive development aspects of the disease

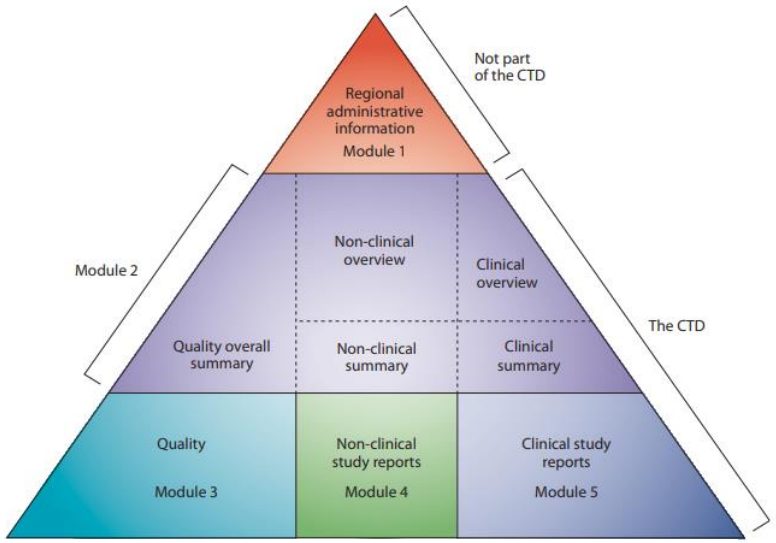
Egetis intends to submit a marketing authorisation application for Emcitate® to the European Medicines Agency based on existing clinical data

- *Egetis concludes, based on recent regulatory interactions, that available Triac Trial I data together with recently published long-term data are sufficient for a Marketing Authorisation Application in Europe*
- *Having all clinical data required for regulatory submission already at hand significantly reduces the remaining risk for Emcitate*
- *Revised submission timelines will be communicated as soon as all parts of the regulatory dossier are confirmed*
- *Egetis will host a webcast today at 15:00 CET (9:00am ET)*

Stockholm, Sweden, December 13, 2021 - Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) today announced that after a pre-submission meeting held last week with concerned European regulatory agencies (EMA's Rapporteur and Co-Rapporteur), the Company concludes that the clinical data from the Triac Trial I (Groeneweg et al. 2019), together with the data from long-term treatment with Emcitate (tiratricol) for up to six years in 67 patients (van Geest et al. 2021) will be sufficient for a regulatory review of a Marketing Authorisation Application (MAA) to the European Medicines Agency for the treatment of monocarboxylate transporter 8 (MCT8) deficiency. Thus, all clinical data necessary for regulatory submission is already available. The ongoing Triac Trial II will continue to further establish the effects of early intervention on the neurocognitive development aspects of the disease.

"We are delighted with the outcome of the pre-submission meeting, giving us a clear path to our MAA submission, and subsequent regulatory review, based on existing clinical data. Having all clinical data required for regulatory submission already at hand significantly reduces the remaining risk for Emcitate and could also potentially enable an earlier submission in Europe than we had previously expected. This is a substantial opportunity for us and the European patients suffering from MCT8 deficiency. In parallel, as part of our efforts to make Emcitate available as soon as possible, we continue our dialogues with regulatory authorities in other jurisdictions to obtain their views on the available clinical data and its implications for regulatory submissions" said Nicklas Westerholm, CEO, Egetis Therapeutics.

Content in *Emcitate* MAA submission



Common technical document

- Regulatory submissions in major regions contain the same type of key information on Efficacy, Safety and Quality
  - presented in a common format (called CTD - Common Technical Document)

Key components of regulatory dossier

Clinical data ✓	Non-clinical data ✓	General items ✓	CMC ✓

CMC: Chemistry, manufacturing and controls

Treatment effects on T3 levels in MCT8-deficiency could provide a basis for marketing approval in the US – NDA targeted in mid 2024

- FDA acknowledges that a treatment effect on T3 levels and the manifestations of chronic thyrotoxicosis in MCT8-deficiency could provide a basis **for marketing approval** also in the US.
- A small, 30-day, placebo-controlled study in 16 treated patients, to be identified from treatment naïve patients and through our existing named patient program, will be conducted to **verify the results on T3** levels seen in previous clinical trials and publications in a randomized **controlled** setting.
- An **NDA** in the US is targeted to be submitted in **mid 2024** under the Fast Track Designation.
- A major step towards marketing authorization and increases the likelihood of success for *Emcitate* and the probability to receive a US Rare Pediatric Disease **Priority Review Voucher (PRV)**.

Egetis concludes that demonstrating treatment effects on T3 levels in MCT8-deficiency could provide a basis for marketing approval for Emcitate® in the US

- *Emcitate® (tiratricol) is the first potential treatment of MCT8 deficiency, a rare genetic disease with high unmet medical need and no available treatment*
- *In recent positive regulatory interactions, FDA acknowledges that a treatment effect on T3 levels and the manifestations of chronic thyrotoxicosis in MCT8-deficiency could provide a basis for marketing approval also in the US.*
- *An NDA in the US is targeted to be submitted in mid-2023 under the Fast Track Designation.*
- *A small, 30-day, placebo-controlled study in 16 treated patients, to be identified through the existing named patient program, will be conducted to verify the results on T3 levels seen in previous clinical trials and publications in a randomized controlled setting*
- *This is a major step towards a marketing application and increases the likelihood of success for Emcitate and the probability for Egetis to receive a US Rare Pediatric Disease Priority Review Voucher (PRV).*
- *Egetis will host a webcast today at 15:00 CET (9:00am ET)*

Stockholm, Sweden, January 18, 2022 - Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) (the "Company") today announced that in recent regulatory interactions, the US Food and Drug Administration (FDA) acknowledges that demonstrating a treatment effect on thyroid hormone T3 levels and the manifestations of chronic thyrotoxicosis could provide a basis for marketing approval also in the US. Consequently, the Company now has an aligned regulatory strategy for EU and US. The Company intends to submit a New Drug Application (NDA) in the US for Emcitate® (tiratricol) for the treatment of monocarboxylate transporter 8 (MCT8) deficiency in mid-2023 under the Fast Track Designation granted by the FDA in October 2021. This follows the announcement in December 2021 of intention to submit the Marketing Authorisation Application (MAA) for Emcitate to the European Medicines Agency (EMA) based on existing clinical data on the manifestations of chronic thyrotoxicosis in MCT8 deficiency.

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

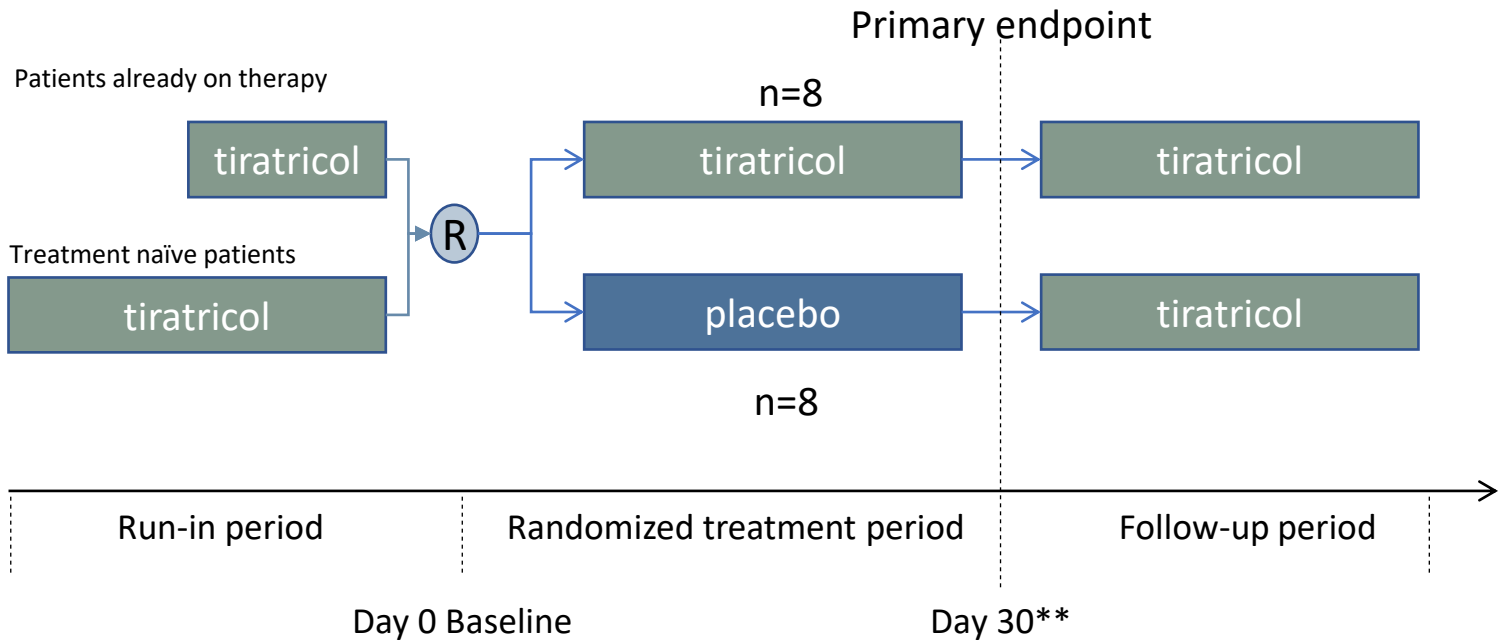
Pivotal randomized placebo-controlled trial for NDA submission

Primary endpoint	• Proportion of participants who meet the rescue criterion (serum total T3 > ULN) during the 30-day double-blind Randomized Treatment Period
Secondary endpoints	• Change in cardiovascular variables • Change in serum thyroid hormone variables
Description	• Double-blind, randomized, multicenter placebo-controlled study • Participants with stable maintenance treatment with *Emcitate* or treatment naïve patients • Design agreed with FDA; Clinicaltrials.gov identifier: NCT05579327
# of patients	• 16 patients, > 4 years of age • Patients from NPU program and treatment naïve patients
Timetable	• First site initiated Q2 2023 • First patients recruited Q3 2023 • Completion expected H1 2024

ReTRIACt – A randomized placebo-controlled withdrawal study designed to show an effect on the proportion of patients needing rescue treatment

Verifying previous results in single arm Triac Trial I and a real-world cohort study

- A 30-day, placebo-controlled withdrawal study in 16 treated patients, to **verify the results on T3** levels seen in previous clinical trial and publications - but in a randomized **controlled** setting
- Design agreed with FDA (no change)
- The study allows for inclusion of both patients that are already on therapy, as well as patients that are previously treatment naïve
- Treatment naïve patients require a longer run-in period to stabilize T3 levels around normal range before randomization
- Thus, a higher proportion of treatment naïve patients will lead to an extended study duration.



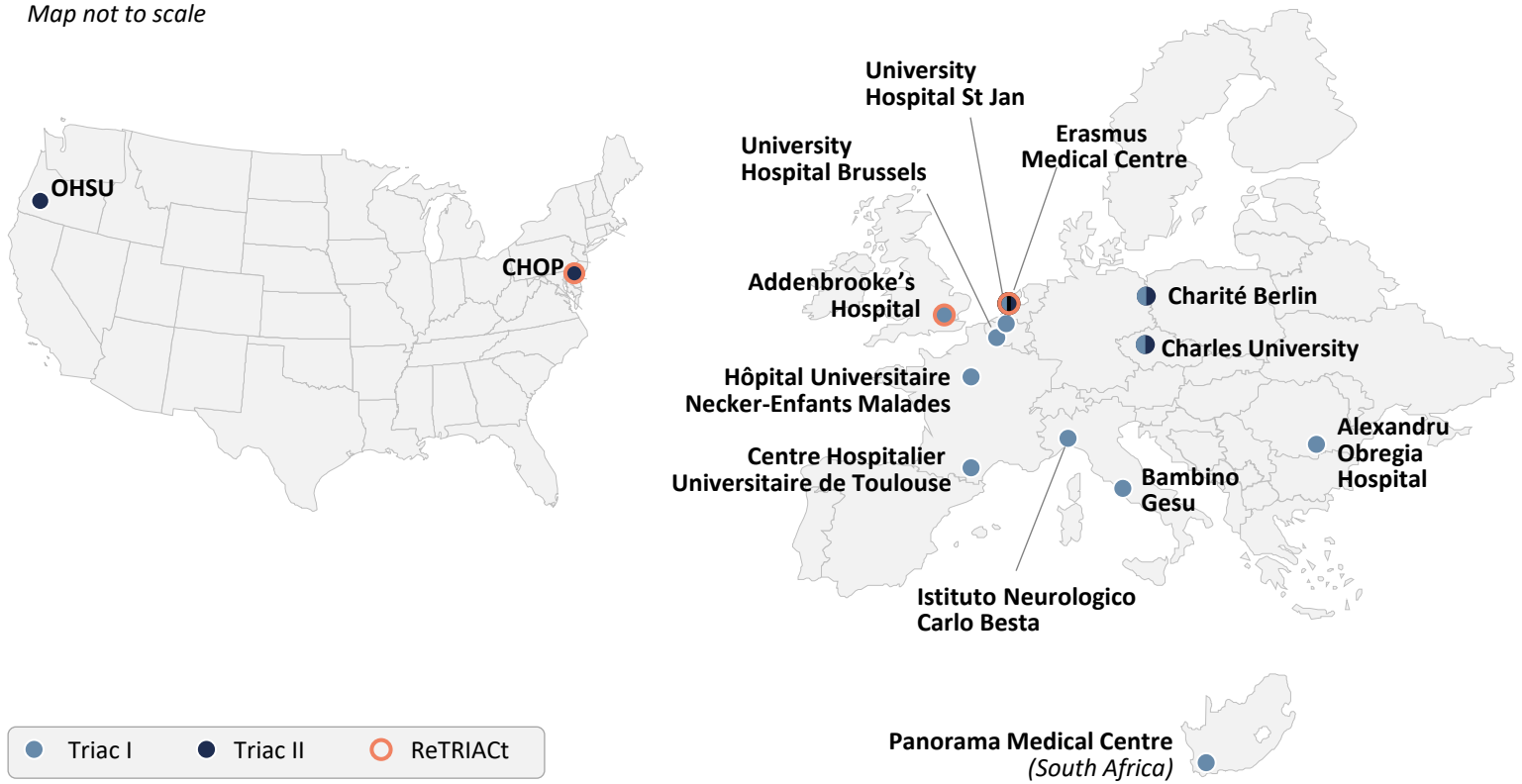
Primary endpoint: Proportion of participants who meet the rescue criterion (T3>ULN*) during the 30-day double-blind randomized treatment period

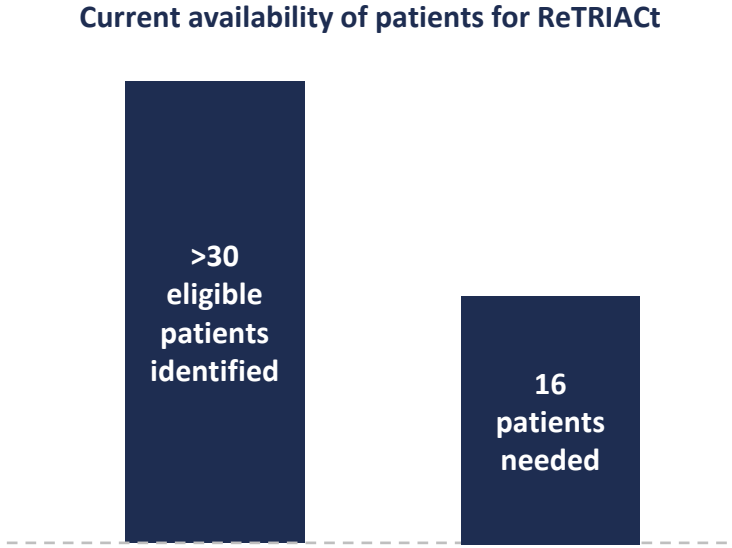
* ULN: Upper Limit of Normal

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

Availability of patients at study sites in ReTRIACt

Map not to scale





All 3 sites in ReTRIACt participated in prior Triac Trial I and/or ongoing Triac Trial II

Triac I study sites include: Addenbrooke's Hospital (Cambridge, UK), Alexandru Obregia Hospital (Bucharest, Romania), Bambino Gesu (Rome, Italy), Centre Hospitalier Universitaire de Toulouse (Toulouse, France), Charité Berlin (Berlin, Germany), Charles University (Prague, Czech Republic), Erasmus Medical Centre (Rotterdam, Netherlands), Hôpital Universitaire Necker-Enfants Malades (Paris, France), Istituto Neurologico Carlo Besta (Milan, Italy), Panorama Medical Centre (Panorama, South Africa), University Hospital Brussels (Brussels, Belgium) and University Hospital St Jan (Brugge, Belgium).
Triac II study sites include: Children's Hospital of Philadelphia (Philadelphia, Pennsylvania), Charité Berlin (Berlin, Germany), Charles University (Prague, Czech Republic), Erasmus Medical Centre (Rotterdam, Netherlands) and OHSU (Portland, Oregon).
ReTRIACt study sites include: Addenbrooke's Hospital (Cambridge, UK), Children's Hospital of Philadelphia (Philadelphia, Pennsylvania) and Erasmus Medical Centre (Rotterdam, Netherlands).

Triac Trial II fully recruited: to establish effects of early intervention on neurocognitive development

Market approval not dependent on Triac Trial II data

Primary Objective	• Confirm findings from Triac Trial I in youngest age group • Improvement in neurocognitive development as measured by GMFM[1] and BSID-III[2] compared to natural history controls
Secondary Objective	• Achievement of motor milestones (e.g. hold head, sit independently) • Normalization of thyroid hormone function tests and markers of thyrotoxicosis
Description	• Open label, multi-centre trial in very young children with MCT8 deficiency • International trial with 10 centres in CZ, DE, IT, UK, FR, NL, US • Design discussed and anchored with EMA and FDA • ClinicalTrials.gov identifier: NCT02396459
# of Patients	• 22 children, 0-30 months of age
Timetable	• First Patient First Visit in Dec 2020, recruitment target met in April 2022 • Results from 96 week read out expected mid 2024 and data is expected to be submitted post-approval to regulatory authorities shortly thereafter and available for HTA interactions • Market approval not dependent on Triac Trial II data

1. Gross motor function measure.
2. Bayley Scales of Infant Development.

Upcoming pipeline milestones

Emcitate®

2022

- ✓ US & EU ODD RTH-b
- ✓ Recruitment completed in Triac Trial II, Q2 2022

2023

- ✓ FPI ReTRIACt for US NDA
- ✓ Filing EU MAA Oct 9, ‘23

2024/25

- Results ReTRIACt for US NDA
- Filing US NDA mid ‘24 under Fast Track Designation
- Results Triac Trial II
- EU approval and launch
- US approval and launch
- US Rare Pediatric Disease Priority Review Voucher

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.
- PRV program prolonged until 2026.
- 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 2021-23 PRVs have been sold ranging from $100m-$110m.

Examples of PRVs sold

Seller	Buyer	Value	Year
Liminal Biosciences	Undisclosed	$105M	2021
Mirum Pharmaceuticals	Undisclosed	$110M	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
Sarepta	Undisclosed	$103M	2023

Under the terms of the acquisition of RTT in Q3 2020, 50% of the potential net proceeds from a sale of a PRV related to Emcitate should be paid as an earnout to the sellers of RTT

2.

Emcitate® - Commercial opportunity

Emcitate®– 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 years[1]
- 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 year[2]

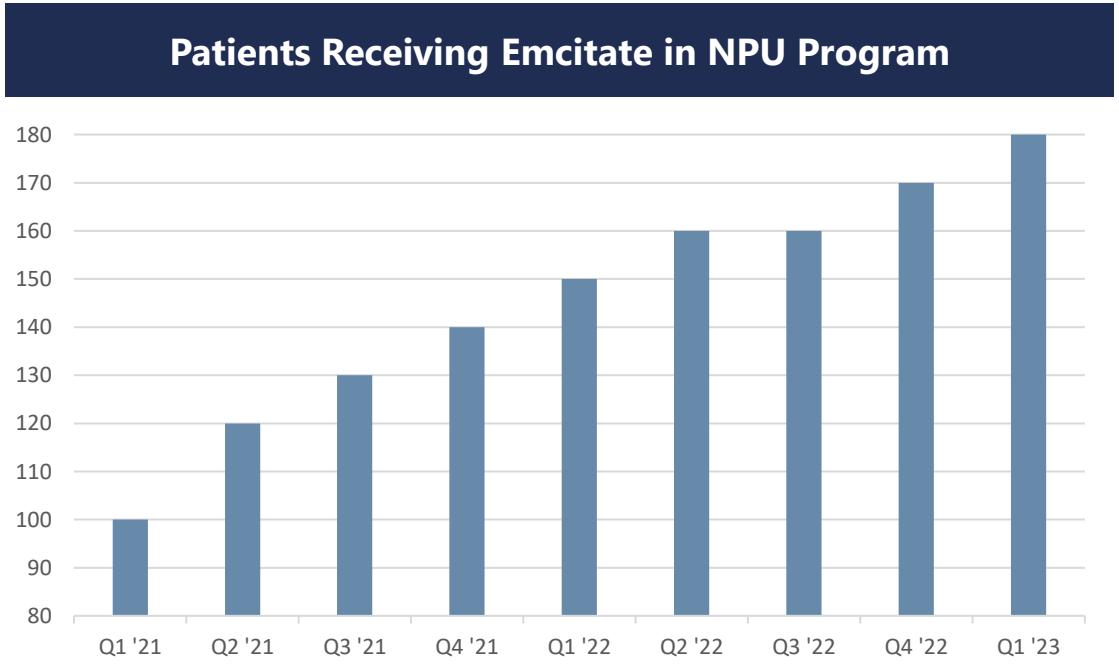
Emcitate holds potential to become the **first approved therapy** to address the root cause of MCT8 deficiency, restore thyroid hormone signaling and thereby **prevent disease progression**, alleviate symptoms and **prolong lives**

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 on a named patient basis

The named patient use (NPU) confirms the significant unmet medical need in MCT8 deficiency and the view on how Emcitate address it

- NPU and compassionate use 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 on a named patient basis, following individual approval from the national medicines agencies, to
  - over 180 patients
  - in over 25 countries



Commercialization of *Emcitate*

Disease area conditions provide opportunity for lean commercialization

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** ✓

Stepwise establishing in-house commercial capabilities

- Preparing for **2024-25 launches** in Europe and US, respectively
- Commercial organization of **40-50** employees at time of launch
- Aiming for rapid access to Emcitate for all **MCT8 deficiency patients**
- Plan to commercialize in rest of world through partners

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

ALEXION

Marianne Berrens-Peijnenburg, NL
Global Head, Medical Affairs

SANOFI GENZYME

Nadia Georges, CH
Global Head, Market Access & Pricing

SANOFI

Peter Verwaijen, NL
Global Head
Marketing & Brand Strategy

ACTELION

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

BIOMARIN

Anny Bedard, US
President Egetis North America

SAREPTA THERAPEUTICS

Shire

John Walsh, US
VP, Medical Affairs North America

Biogen

Kate Sulham, US
VP, Market Access & Pricing

THE MEDICINES COMPANY®

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

VERTEX

Sylvain Forget, FR
GM Southern Europe
(FR, ES, PT, IT, GR)

sobi
Pioneer in Rare Diseases

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.

Disease awareness initiatives are bearing fruit

- Awareness of MCT8 deficiency remains low also among specialists, with a high proportion of patients living without correct diagnosis
- Increasing disease awareness and facilitating diagnostic testing are key strategic imperatives for 2023
- Over 40 new patients identified in the US only this year
- Emcitate is presently being supplied on a named patient basis, following individual approval from the national regulatory agencies, to
  - over **180** patients
  - in over 25 countries
- An Expanded Access Program has been approved in the US, allowing access to *Emcitate* for patients that *do not meet the inclusion criteria in ongoing clinical trials in the US*

DISEASE AWARENESS AND EDUCATION

COLLABORATION WITH PAGs & KOLs

EXHIBIT AT SCIENTIFIC/MEDICAL CONFERENCES

Increasing number of previously undiagnosed and treatment naïve patients are being identified

Aiming for broad access to *Emcitate* for affected families

Payer projects initiated to generate optimal reimbursed price
– No families should pay out of own pocket

- Payers in general **accept higher prices** for orphan drugs compared to traditional drugs and especially if they;
  - Address an **ultra-rare** disease, e.g. prevalence less than 1:50,000 people
  - Target a **severe** disease, i.e. life threatening/debilitating

- ***Emcitate*** fulfills these criteria, no other drugs available or being developed for MCT8 deficiency

☑ 1:70,000 males & even more rare in females

☑ Severe impact on QoL, median survival 35y

The pricing & reimbursement work has started

1. VALUE IDENTIFICATION, POSITIONING & EVIDENCE GENERATION

2. PRICE STRATEGY IMPLEMENTATION & VALUE COMMUNICATION

Aiming for that Emcitate as soon as possible after marketing authorization is financed through country specific reimbursement mechanisms and that no family would have to pay for treatment out of own pocket

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

Key for payer assessments to describe unmet need & quantify burden of MCT8 deficiency

- The **impact of MCT8 deficiency on patients and caregivers is underreported**
- Significant **clinical and economic burden**, both direct and indirect, which will be described and quantified
- Currently **generating data** for payers to answer the question *"What is the burden of MCT8 deficiency for patients & their caregivers?"*
  - **Vignette study** – Involving treating physicians to derive utility values for a defined range of MCT8 deficiency health states, suitable for cost-effectiveness analysis
  - **Caregiver study** – Generate burden of disease data (costs & QoL) from caregivers

Example of Caregiver burden of disease publication in CLN2

Original Article

The Challenges of Living with and Caring for a Child or Children Affected by Neuronal Ceroid Lipofuscinosis Type 2 Disease: In-Depth Family Surveys in the United Kingdom and Germany

Angela Schulz[1], Mohit Jain[2], Thomas Butt[2], Rachel Ballinger[3], Lina Eliasson[3], Jake Macey[3], Tessa Peasgood[3], Andrew Olaye[3], Irini-Alexia Terzakis-Snyder[4], Iris Dyck[5] and Andrea West[6]

Abstract

Limited research has investigated the challenges faced by families caring for children with neuronal ceroid lipofuscinosis type 2 (CLN2) disease. Face-to-face, mixed-method, in-depth surveys were conducted with 19 families (23 children) in the UK (n=9) and Germany (n=10) to assess the impact of caring for children with CLN2 disease, using national wellbeing and quality of life (QoL) measures. Primary (n=19) and secondary (n=10) caregivers, adult siblings (n=2), and child siblings (n=2) were included. Caregivers reported reduced health-related QoL compared with age and gender-matched controls (mean utility scores 0.08 and 0.11 lower in Germany and the UK, respectively). Hours of caregiving were significantly higher relative to that provided to a child of normal health, with stress, back pain, and reductions in sleep being recorded. Lower life satisfaction and happiness with partners were also reported, along with significant financial burden. Those caring for children in the late stage of disease were more greatly impacted than those with children in the rapidly progressive stage, or who were bereaved. The results of this study make clear the importance of emotional and practical support for caregivers and siblings coping with CLN2 disease.

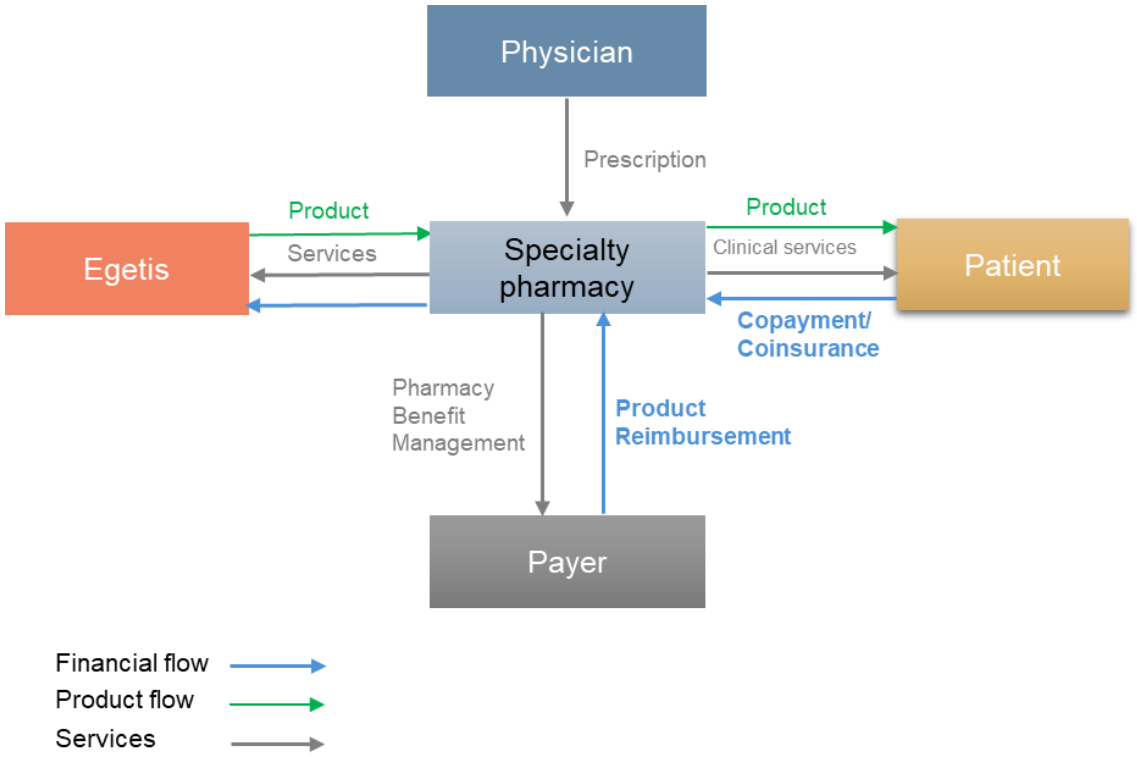
Keywords:
Rare disease, Family, Disease burden, CLN2 disease, Batten disease.

Background

Neuronal ceroid lipofuscinosis type 2 (CLN2) disease is part of a group of rare, progressive, degenerative, neurometabolic disorders called neuronal ceroid lipofuscinoses (NCLs; also known as Batten disease) [1–3]. This rare, autosomal recessive disorder is caused by pathologic variants (mutations) in the *TPP1* gene (also known as the *CLN2* gene) which lead to deficiency of an enzyme called tripeptidyl peptidase 1 (TPP1). The deficiency of TPP1 results in abnormal levels of storage materials in neurons and other cells, and ultimately neuronal loss in the central nervous system [4–5].

Onset of CLN2 disease generally begins when the child is aged 2–4 years, followed by a rapid decline in motor and language abilities [6]. The first symptoms frequently reported are seizures; however, a significant delay in language development often precedes the first seizure [7]. Affected children have usually had

Exclusive Distribution Model Through Speciality Pharmacy is Preferred option for Rare Disease



- Insurance resolution and contracting
- Prior authorization support
- Appeals
- Dedicated case managers
- Improves patient experience and outcomes
- Patient Assistance and Copay Support

US Pricing & Reimbursement

Relatively straight forward for ultra-orphans with key focus on rarity and severity of disease

Analogues selected based on:

- Rarity (ultra-orphan)
- Paediatric
- No treatment options
- Life-long treatment
- Disease severity

Emcitate's value drivers confirmed by US payer research:

*"You have all the things here: **terrible condition, ultra rare, deteriorating cognition, etc**"*

Chief Medical Officer
Commercial Payer

*"The product gets paid for because they are **kids** and they need outcomes"*

Medical Director
Paediatrician
Medicaid

*"If **FDA approves** this treatment, **we will cover it.**"*

Medical Director,
Paediatrician
Medicaid

US Payer Analogues

	Exondys® *anti-sense oligonucleotide*	**Ravicti®** *Small molecule*	**Oxlumo®** *iRNA*	**Brineura®** *Recombinant enzyme*
Disease	Duchenne Muscular Dystrophy (13% of population)	Urea Cycle Disorders	Primary Hyperoxaluria	CLN2
Rarity - less than 1:50,000 people	✓	✓	✓	✓
Severity – life threatening/debilitating	✓	✓	✓	✓
US gross annual treatment cost	$750k	$750k	$500k	$750k

3.

Paracetamol/Acetaminophen overdose and clinical experience with Aladote

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

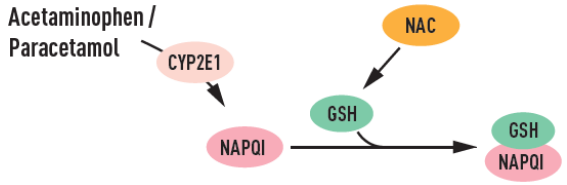
- **Aladote®** aims to become **a new standard of care** for patients with increased risk for liver injury in combination with NAC

Orphan drug candidate

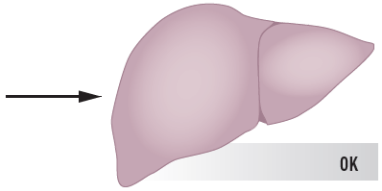
with clear scientific and mechanistic rationale

Early presenters (<8h)
NAC treatment effective against liver injury

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

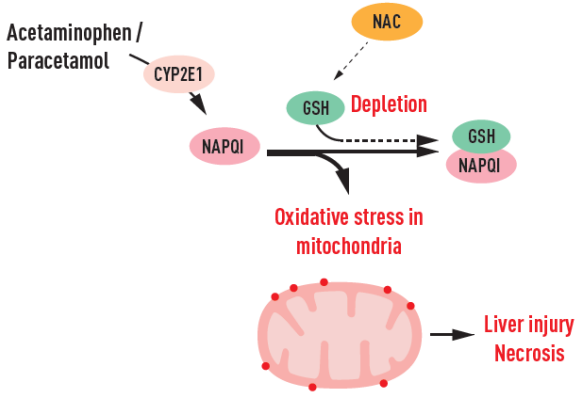


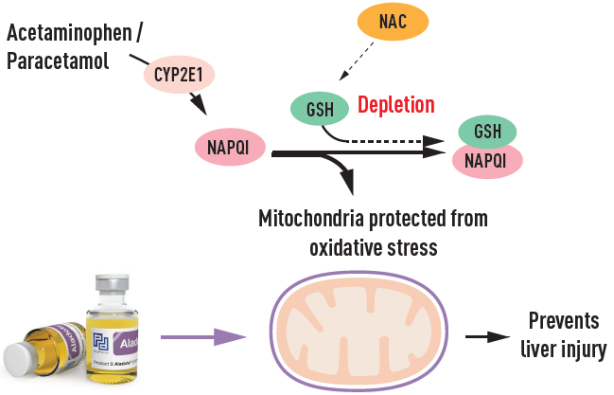
- In most cases NAC effectively prevents liver injury i.e. limited need for Aladote®



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**

Source: Akakpo et al. 2020, Burke et al. 2010.

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

Overview of completed Phase Ib/IIa

Primary objective and results	• Met primary endpoint of safety tolerability in the combination of Aladote® and NAC • Results presented at the 58th Annual Meeting of the Society of Toxicology, EASL ILC in April, Vienna and published in Lancet's journal EBioMedicine in 2019 • Presented 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	• 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	• An open label, rising-dose, randomized study exploring safety and tolerability of Aladote® co-treatment with NAC • ClinicalTrials.gov identifier: NCT03177395
# of patients	• Single ascending dose study in 3 dosing cohorts of 8 patients (N=24) as add-on to NAC regime
Timetable	• 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

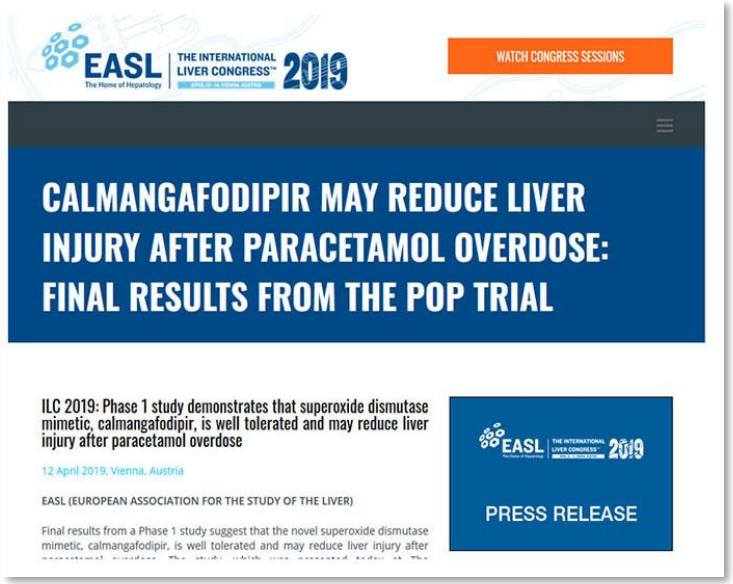
ELSEVIER

EBioMedicine
Published by THE LANCET

Principal results of a randomised open label exploratory, safety and tolerability study with calmangafodipir 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], Wilna Oosthuyzen [a], Robert J. Lee [b], Christopher J. Weir [b], Dennis Henriksen [d], James W. Dear [a,*], On behalf of the POP Trial Investigators [1]

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



Positive proof-of-principle Phase Ib/IIa results

Indicates that Aladote may reduce liver injury

Safety & tolerability

Event	NAC alone	NAC + 2 µmol/kg Aladote	NAC + 5 µmol/kg Aladote	NAC + 10 µmol/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%)

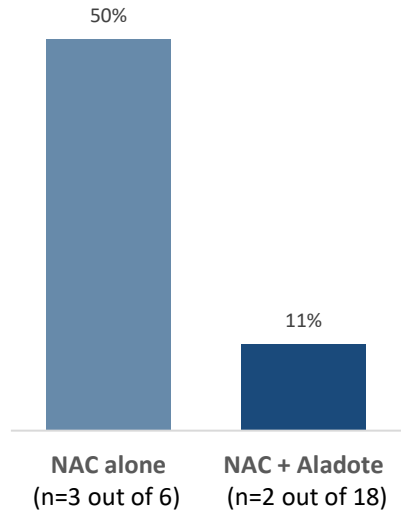
- Met primary endpoint of safety tolerability in the combination of Aladote® and NAC
- No AE or SAE probably or definitely related to Aladote®

Liver injury – ALT[1] pre-defined secondary outcome

Event	NAC alone	NAC + 2 µmol/kg Aladote	NAC + 5 µmol/kg Aladote	NAC + 10 µmol/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

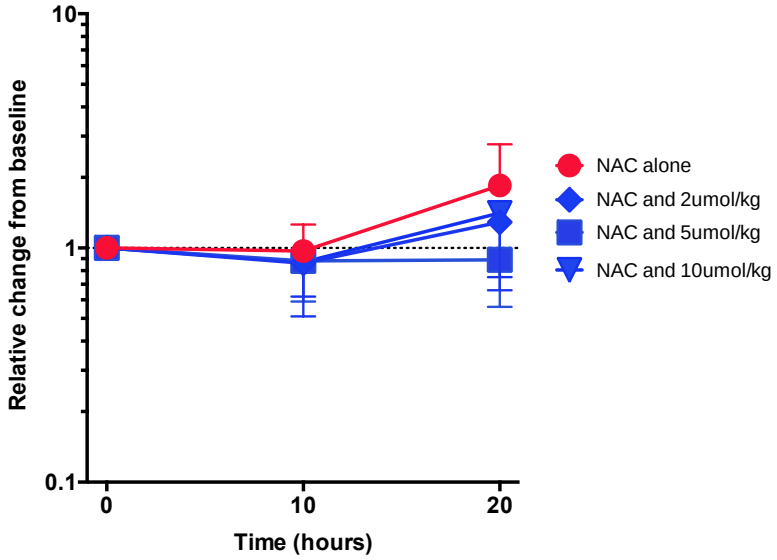
% of patients needing additional NAC infusions after planned 12h NAC infusion



Note: (1) Alanine transaminase (ALT) is a transaminase enzyme found in plasma and in various body tissues especially the liver's hepatocytes. Serum ALT is commonly measured clinically as part of a diagnostic evaluation of hepatocellular injury, to determine liver health

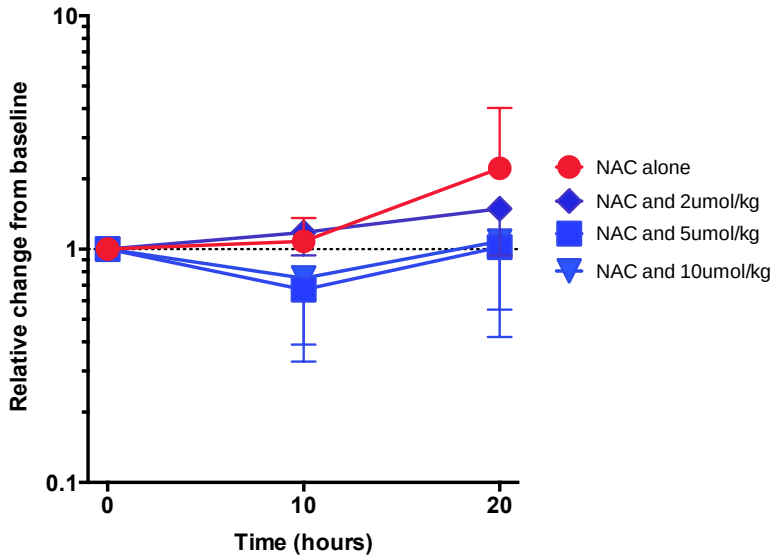
Aladote® demonstrates consistent results of reduced liver injury as measured by exploratory biomarkers

K18



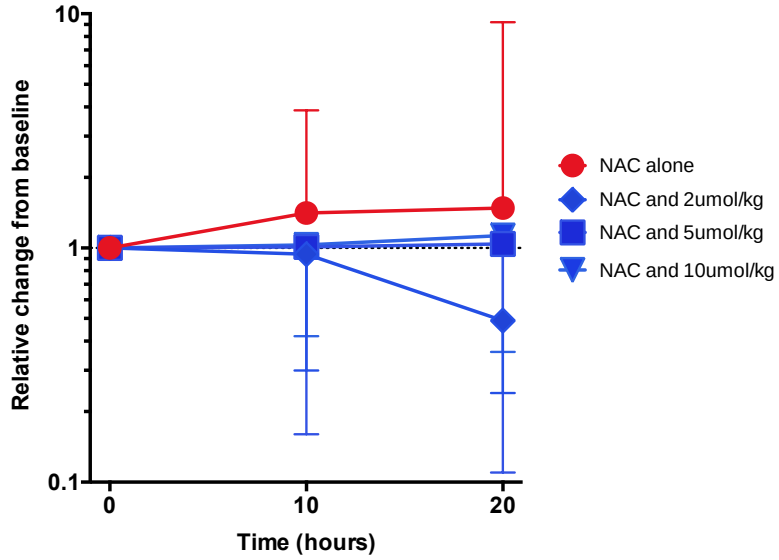
K18 is a measure of cell death and correlate with peak ALT activity during the hospital stay

ccK18



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

miR-122



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* submissions have been completed

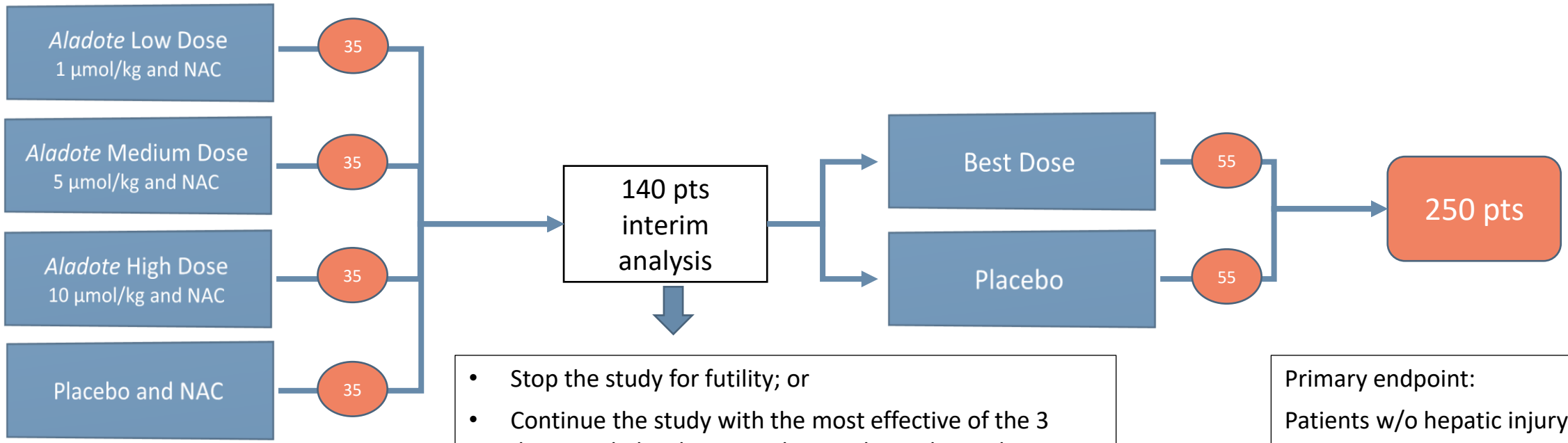
ALBATROSS: Phase IIb/III study for US/EU regulatory submission

Patient population	• 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	• Approved 21 hours NAC regimen
Treatment groups	• 4 groups in combination with NAC: *Aladote* high dose; *Aladote* middle dose; *Aladote* low dose; Placebo
Initiation of active treatment	• 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	• 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	• 250 patients planned
Efficacy endpoints	• 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	• EU, UK and USA

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

2022

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

tbc

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

tbc

- Interim analysis
- Recruitment completed and topline results

tbc

- 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 POD[1]
- The POD Emergency Department and inpatient cost is approximately USD 13-40k[1]
- The average POD inpatient length of stay is 3.1 days with a variance of +4.4 days for the most severe cases[1]
- US liver transplant costs USD 125-473k[1]

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

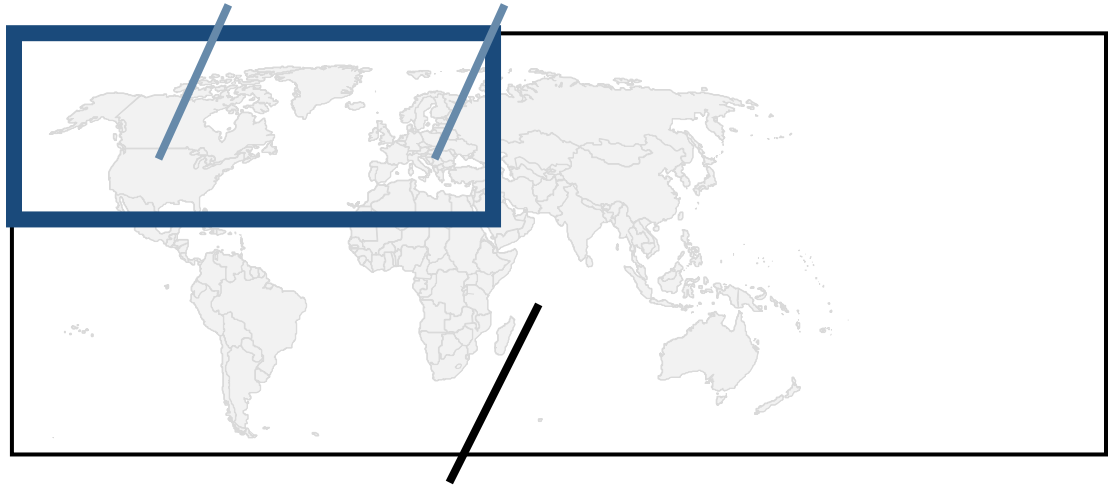
- **Addressing unmet medical need** ✓
- **Leading KOL support** ✓
- Centralized**, focused target groups** of **specialists** eager to improve care ✓
- Treatment choice **highly protocol driven** ✓
- **No competition** ✓

Addressing life-threatening condition

- Anologue 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 hydrocloride	Praxbind	Andexxa/Ondexxya	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

1. **Orphan drug designation is awarded to products targeting limited disease populations[1]**
2. **More than 7,000 known rare diseases**
3. **Approx. 10% of the general population may be affected by a rare disease**
4. **Substantial unmet medical need for patients, only 5% of rare diseases have an approved therapy**

Development
- Less extensive clinical trials
- Agile and faster development process
- Lower costs
- Lower development risk

Registration
- Free regulatory advice
- Reduced fees
- Expedited review
- Market exclusivity

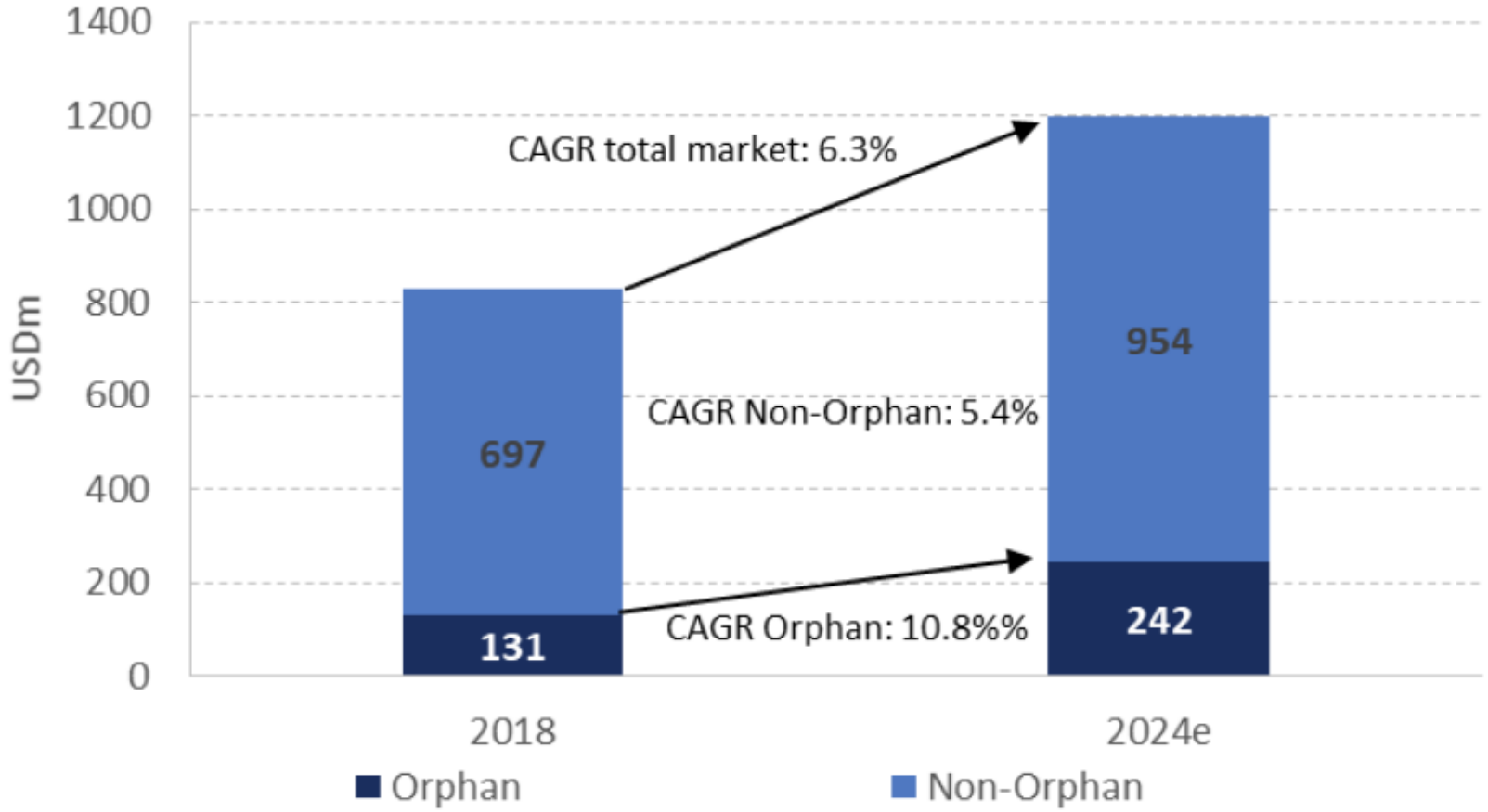
Market
- No or few competitors
- Highly focused target groups
- Premium pricing

Well-defined patient populations with substantial unmet medical need

Note: (1) Populations of less than 5/10,000 inhabitants in the EU or <200,000 inhabitants in the US

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.



Source: EvaluatePharma, Pareto.

5.

Summary

EGTX – 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 and consistent 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 market in 2024, already passed most of typical drug development risks
  - All clinical data necessary for regulatory approval in EU already at hand – Submitted October 9, 2023
  - A small and short trial reconfirming the effect on biomarker T3 under way to complete the US dossier - Submission mid 2024
- Significant market opportunity with potential for premium orphan drug pricing
- Eligible for priority review voucher upon US approval, which can be sold for ~100 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 submitted** on **October 9, 2023,** based on existing clinical data
- **NDA** in **mid 2024** under fast-track designation, after conducting a 30 days placebo-controlled study (ReTRIACt) in 16 patients to verify the results on T3
- **Triac Trial II fully recruited**; to establish the effects of early intervention on **neurocognitive** development, previously seen in Triac Trial I. Results expected mid 2024
- Over **180 patients** are being **treated** with *Emcitate* on a **named patient basis** – Expanded Access Program implemented 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

Upcoming pipeline milestones

Emcitate®

2022

- ✓ US & EU ODD RTH-b
- ✓ Recruitment completed in Triac Trial II, Q2 2022

2023

- ✓ FPI ReTRIACt for US NDA
- ✓ Filing EU MAA Oct 9, ‘23

2024/25

- Results ReTRIACt for US NDA, H1 ‘24
- Filing US NDA mid ‘24 under Fast Track Designation
- Results Triac Trial II
- EU approval and launch
- US approval and launch
- US Rare Pediatric Disease Priority Review Voucher

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**
   Target *Emcitate* **NDA 2024**
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

~30 FTEs

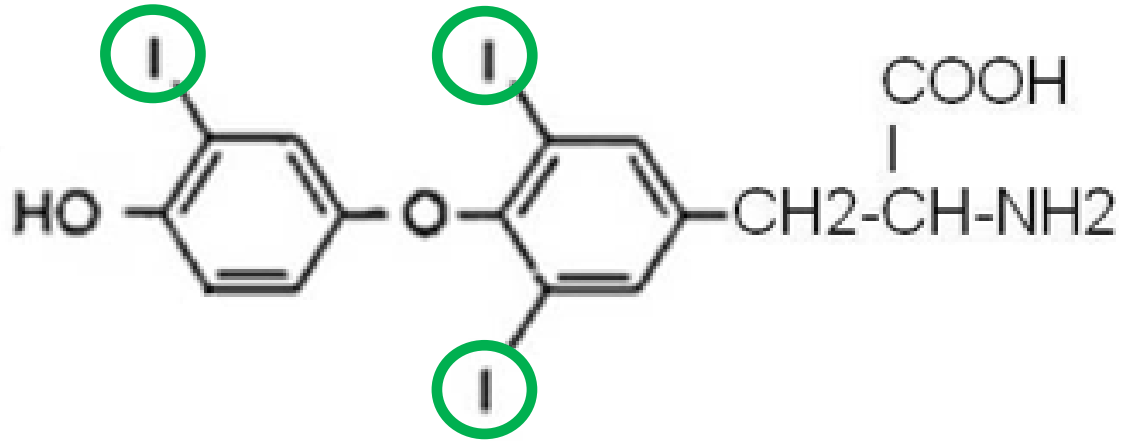
*In-house development of *Aladote* parked until *Emcitate* submissions have been completed

A.

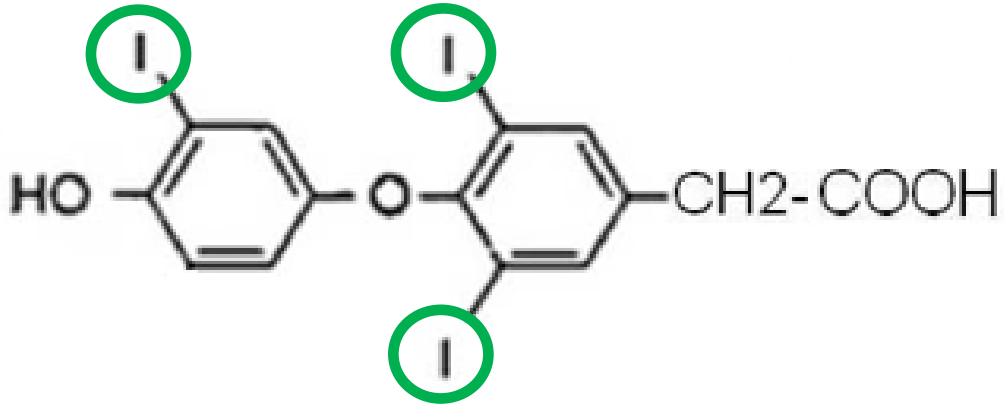
Appendix

Discovery of *Emcitate* (Triac, tiratricol)

T3



Triac
(tiratricol)



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

Preliminary Communication

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

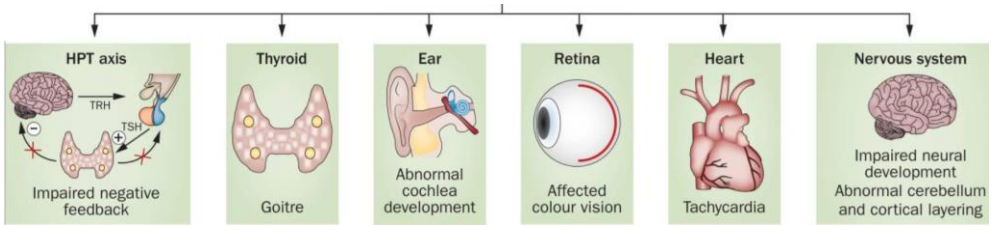
Resistance to Thyroid Hormone type Beta (RTH-β)

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 symptoms[2]
- 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; Holds 212,976 shares
- AstraZeneca 1995-2017
- VP Late-stage development CVMD
- Executive Officer & VP Japan Operations
- Director Investor Relations

Yilmaz Mahshid, PhD

CFO

- Joined 2021; Holds 303,089 shares
- Investment Manager & Controller at Industrifonden
- Sell-side analyst at Pareto & Öhman
- CEO Medivir

Henrik Krook, PhD

VP Commercial Operations

- Joined 2020; Holds 305,999 shares
- Commercial roles at Alexion, Novartis, Roche and Affibody

Karl Hård, PhD

VP IR, Communications & Business Development

- Joined 2022; Holds 0 shares
- Redx Pharma, Optimum Strategic Communications, Kiadis, AstraZeneca

Anny Bedard

President Egetis North America

- Joined 2022; Holds 0 shares
- Commercial leadership roles at Shire and Sarepta

Kristina Sjöblom Nygren, MD

CMO

- Joined 2020; Holds 26,498 shares
- CMO and Head of Development at Santhera
- 18 years at SOBI, Wyeth & AstraZeneca
- Worked as physician in several clinical positions

Christian Sonesson, PhD

VP Product Strategy & Development

- Joined 2017; Holds 36,298 shares
- AstraZeneca 13 years
- Late-stage development expertise from FORXIGA, MOVANTIK, ONGLYZA, BRILINTA & QTERN

Katayoun Welin-Berger, PhD

VP Technical Operations

- Joined 2023; Holds 0 shares
- VP Operations at Calliditas Therapeutics
- Previously at BioGaia and AstraZeneca

Board of directors

Thomas Lönngren

Chair of the board 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
- Board member, Compass Pathways & NDA Group

Peder Walberg

Board member since 2020

- Shares in Egetis: 33,776,221
- Founder and CEO of Rare Thyroid Therapeutics
- MD and BSc in international economy and business administration, Uppsala University
- Other assignments: Board Member, Immedica
- Previous assignments: Founder & CEO, Medical Need; Head, Business Development & Strategy, Swedish Orphan & SOBI; BoD, Wilson Therapeutics; identified decuprate for treatment of Wilson disease

Gunilla Osswald

Board member since 2017

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

Mats Blom

Board member since 2021

- 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

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

#	Owner	EGTX	Capital	Votes
1	Frazier Life Sciences	38675501	13,22%	13,22%
2	Peder Walberg	33776221	11,54%	11,54%
3	Peter Lindell	30007682	10,26%	10,26%
4	Fjärde AP-fonden	21404690	7,32%	7,32%
5	Avla Holding AB	17668330	6,04%	6,04%

- **Cash position:** SEK 179M (~EUR 15M)*
- **Private placement** Oct 10, 2023: SEK 172M (gross)
- **Number of outstanding shares:** 292.6M
- **MCap:** ~SEK 1,6 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
** At June 30, 2023 (Q2 2023 report); ** October 20, 2023*

EGETIS THERAPEUTICS

Acquisition of Rare Thyroid Therapeutics on 5 November 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%)

Note: (1) Royalties of 10% on Emcitate® net sales to Erasmus Medical Centre;

EGETIS THERAPEUTICS

Thank you!

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