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# Corporate presentation

**January 2023**

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

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# Agenda

1. An integrated orphan drug company, focusing on late-stage development for commercialization
2. Emcitate®
    - MCT8-deficiency and 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

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# 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. Target **MAA/NDA** submissions:
   *Emcitate* **2023** and *Aladote* **2025**
3. Highly attractive **orphan drug segment** with potential **>$1Bn annual sales opportunity**
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

# Building a sustainable orphan drug company

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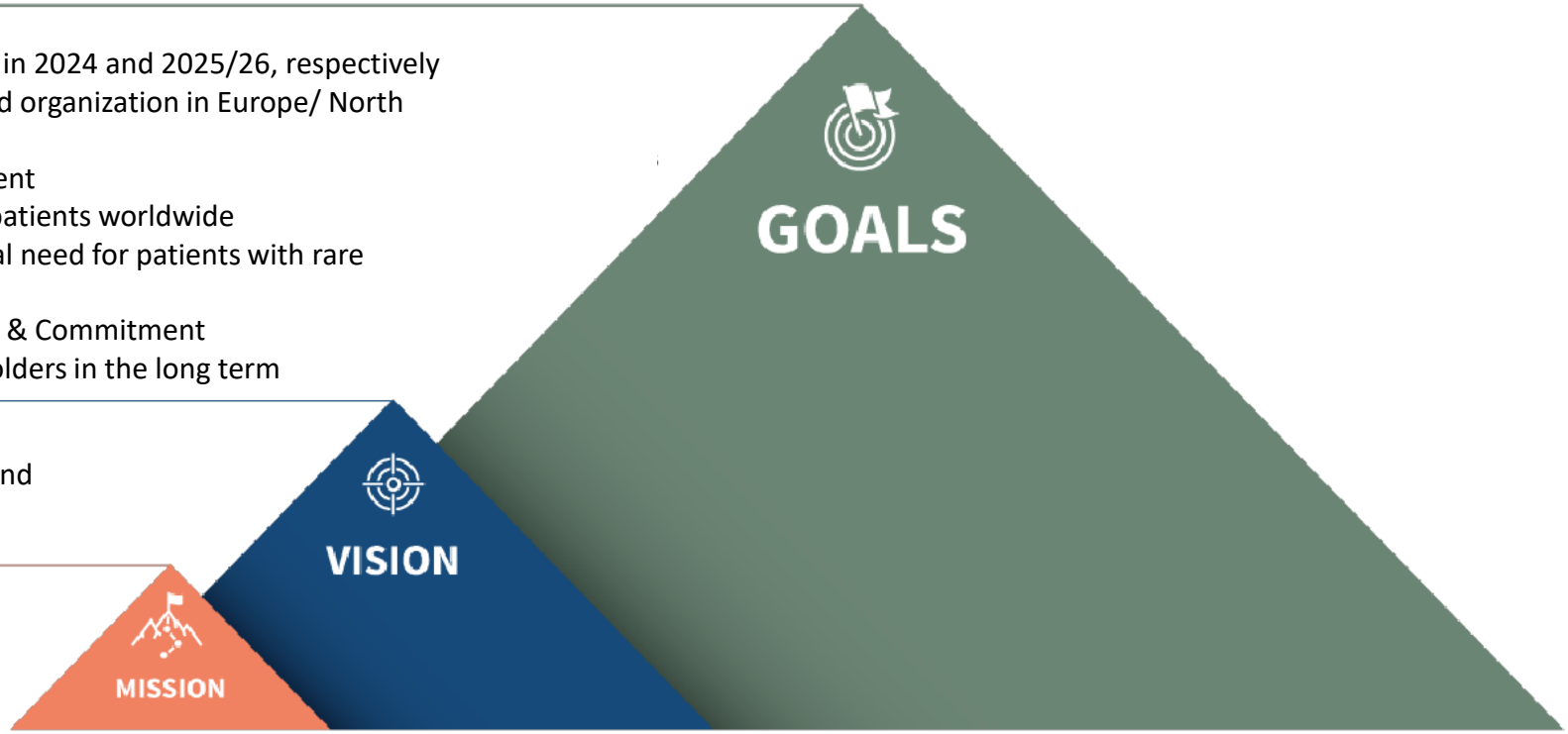
- Successfully develop *Emcitate* and *Aladote* for market approvals in 2024 and 2025/26, respectively
- Commercialize *Emcitate* and *Aladote* through an inhouse focused organization in Europe/ North America and partnerships in RoW
- Realize the full potential of our products via life-cycle management
- 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

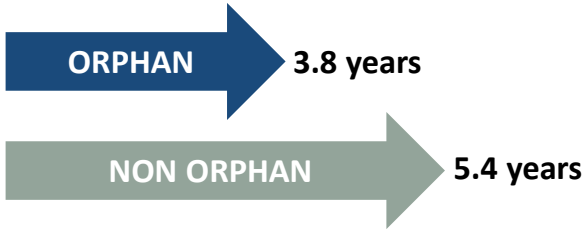
To create value for patients, society and shareholders by developing and providing a portfolio of unique products for the treatment of rare diseases with substantial medical need



# Orphan drug segment – a highly attractive opportunity

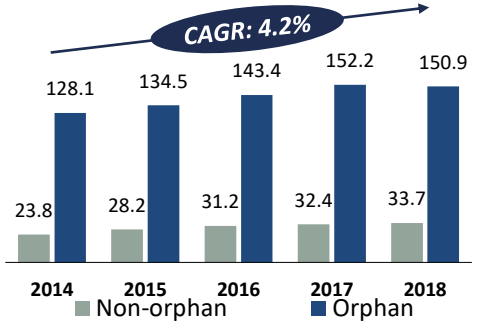
## Shorter clinical development time[1]

**Phase II to launch** Average # of years




## Higher attainable prices[2]

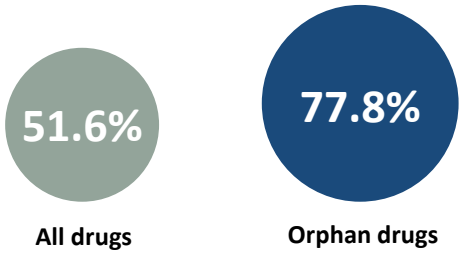
Mean cost per patient and year (USDk)




## Higher probability of success[3]

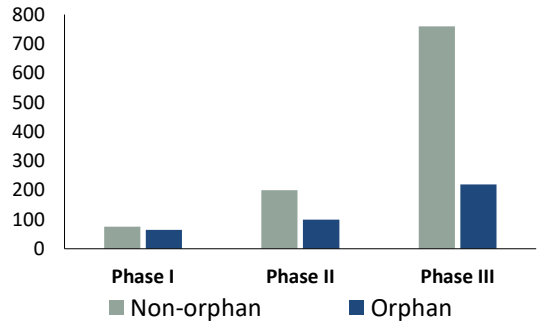
**Phase III to approval**

POS in metabolic/endocrinology indications

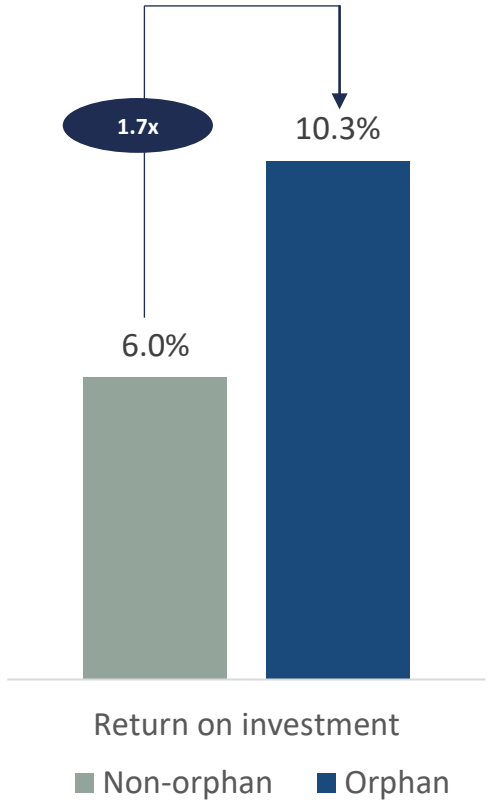



## Fewer patients for clinical trials[4]

Patients per trial



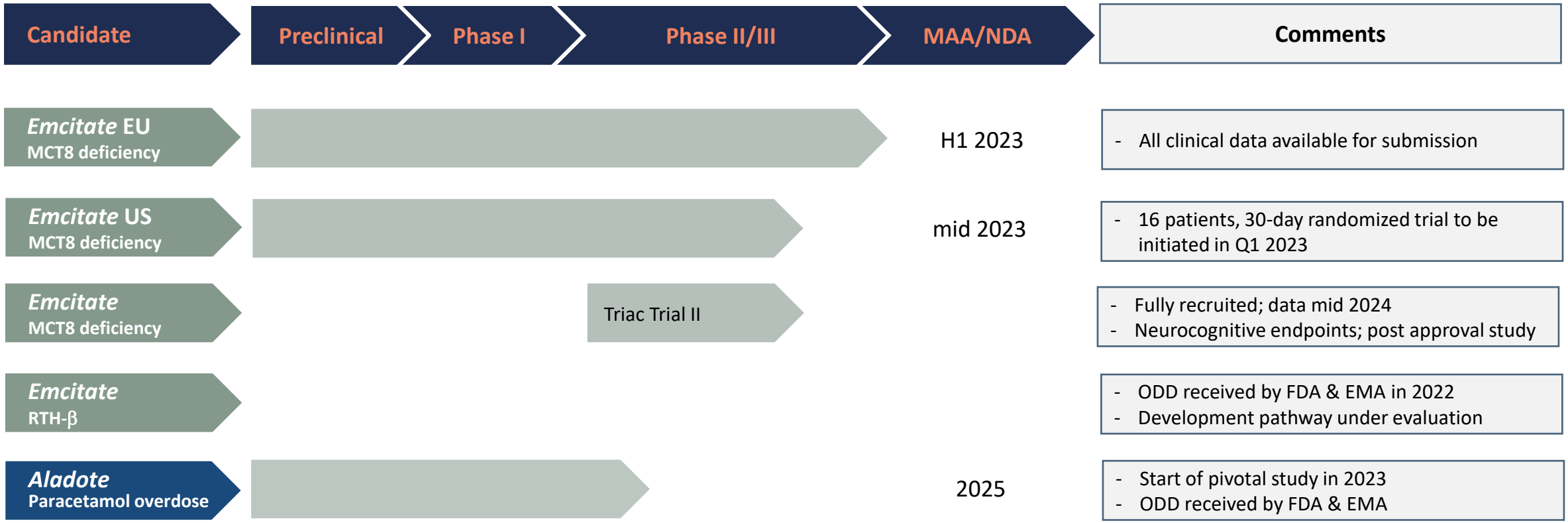

## Orphan drugs attractive returns[5]




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 and US filings in 2023*



# Two highly promising orphan drug candidates

## Emcitate® – *Therapy for MCT8 deficiency*

- MCT8 deficiency affects ~1:70,000 males: high unmet medical need, no available treatment. No competing sponsored products in clinical development
- Orphan Drug Designation in EU & US
- US Rare Pediatric Disease Designation, eligible **for Priority Review Voucher**. Fast track designation granted by FDA
- Triac Trial I (Phase IIb) completed with **significant** and **clinically** relevant effects on **T3 levels** and **chronic thyrotoxicosis**
- Real-world data published **2021 confirms long-term efficacy and safety** of *Emcitate*
- **MAA** in **H1 2023** based on existing clinical data
- **NDA** in **mid 2023**, 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
- More than **160 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, targeting study start in 2023
- No competing products in clinical development

# 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.

*MCT8-deficiency and clinical experience with Emcitate*

# 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 dependant 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

# Orphan drug candidate

*with clear scientific and mechanistic rationale and established safety profile*

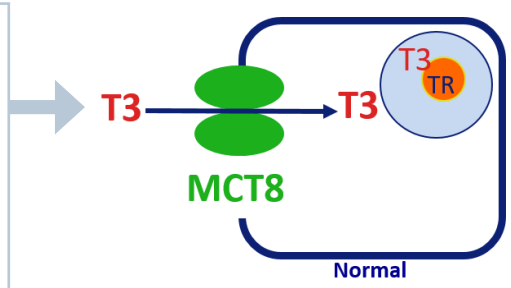
## Difference normal MCT8 and deficiency of MCT8

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

**Normal MCT8** ✔

- Functional thyroid gland producing T3
- Production of functional MCT8

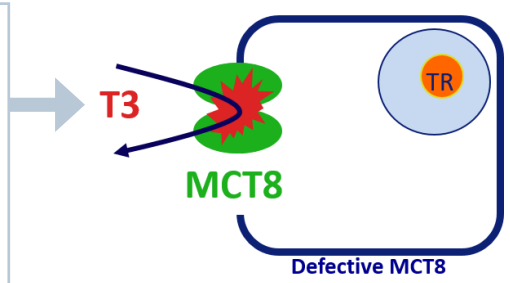
→ T3 cross cell membrane and enters target cell




**Mutated MCT8** ✘

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

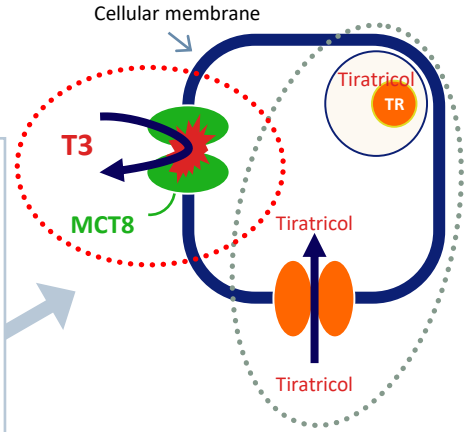
→ T3 cannot cross cell membrane and fails to enter cells




## *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 April 2022, 22 patients. Results expected H1 2024

**Regulatory**

- Orphan drug designation in EU & US, US Rare Pediatric Disease Designation - **eligible for Priority Review Voucher**
- **Fast track designation** granted by FDA
- **Intend to submit MAA to the EMA based on existing clinical data H1 2023**
- US **NDA submission planned mid-2023**: A 30-day, placebo-controlled study in 16 patients will be conducted to verify the results on T3 levels seen in previous clinical trials and publications

**Commercial**

- Est. 10k – 15k MCT8 deficiency patients (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**
- More than **160 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 **10y in EU** (ODD), **7y 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

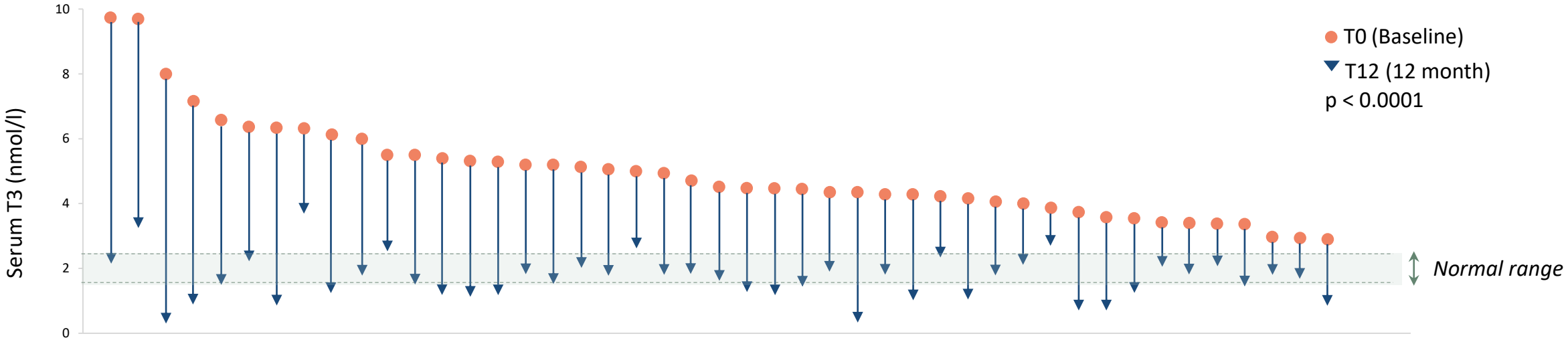
Academic Center for Thyroid Diseases (S Groeneweg MD, Prof R P Peeters MD, M Medici MD, Prof T J Visser PhD, W E Visser MD), Sophia Children's Hospital, Division of Paediatric Cardiology (J M van Beynum MD), Sophia Children's Hospital, Department of Paediatric Neurology (I F M de Coo MD, F K Aarsen PhD, 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 MD, Prof M Polak MD); Department of Paediatric Endocrinology and Genetics, Children's Hospital, Toulouse University Hospital, Toulouse, France

www.thelancet.com/diabetes-endocrinology Published online July 31, 2019 http://dx.doi.org/10.1016/S2213-8587(19)30155-X 1

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*

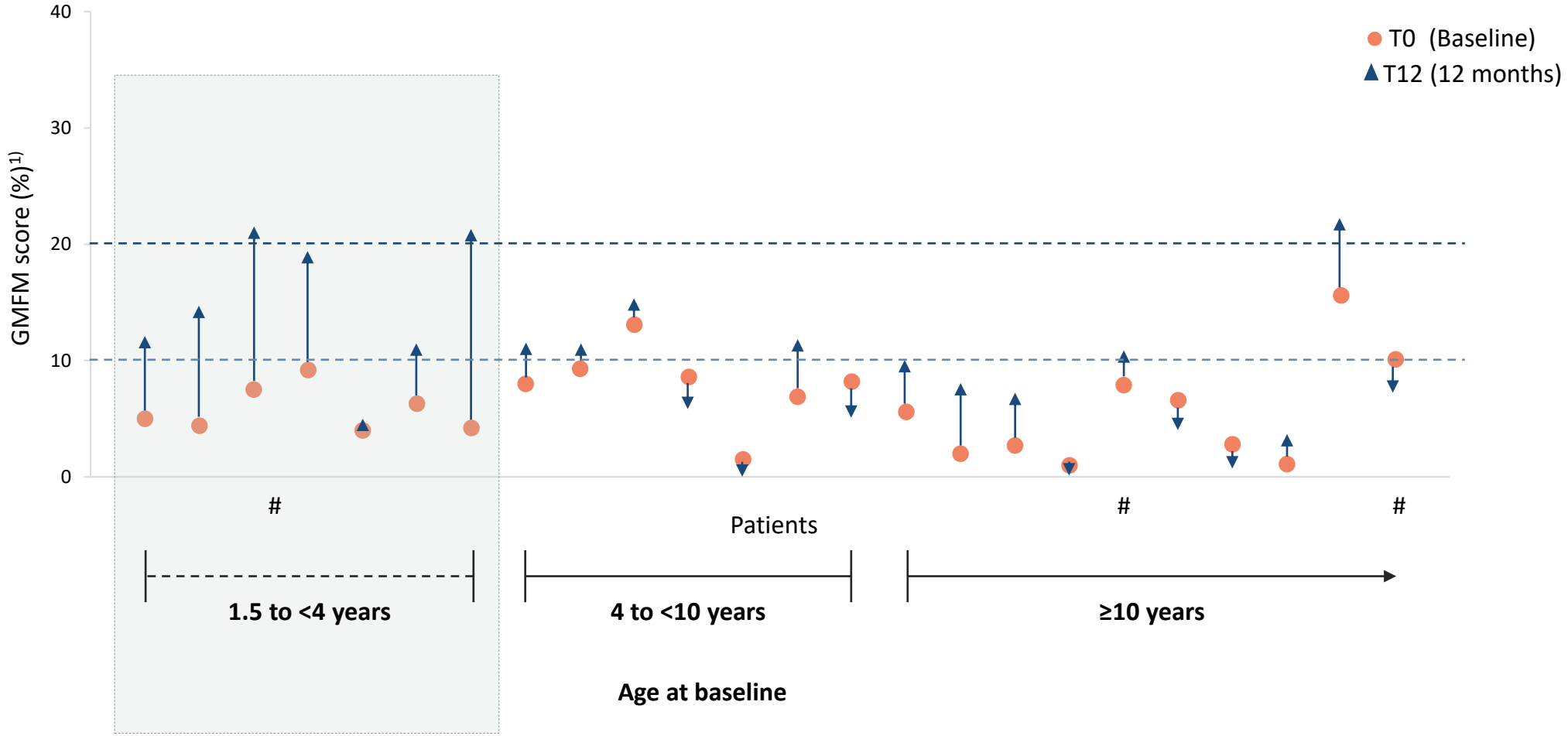



| Endpoints | Baseline mean *(± SD)* | 12 months mean *(± SD)* | Difference in means *(95% CI)* | p-value |
|---|---|---|---|---|
| Serum T3 (nmol/L) | 4.97 (± 1.55) | 1.82 (± 0.69) | -3.15 (-3.62, -2.68) | <0.0001 |
| Weight to age (z score) | -2.98 *(± 1.93)* | -2.71 *(± 1.79)* | 0.27 *(0.03, 0.50)*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

# New data confirms 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

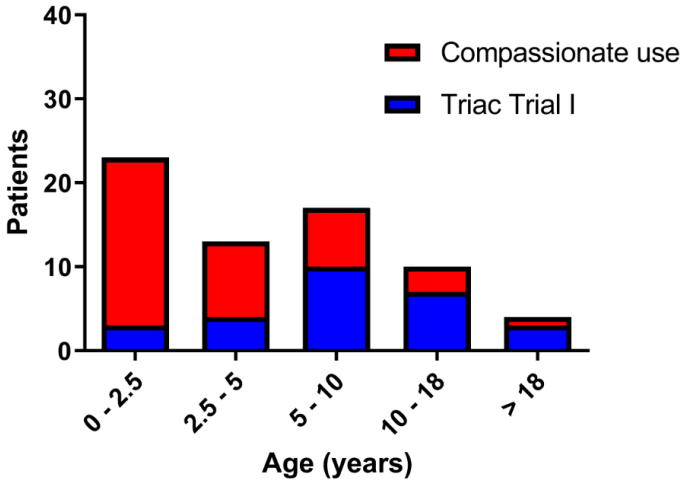
*Long term follow up, up to >6 years*

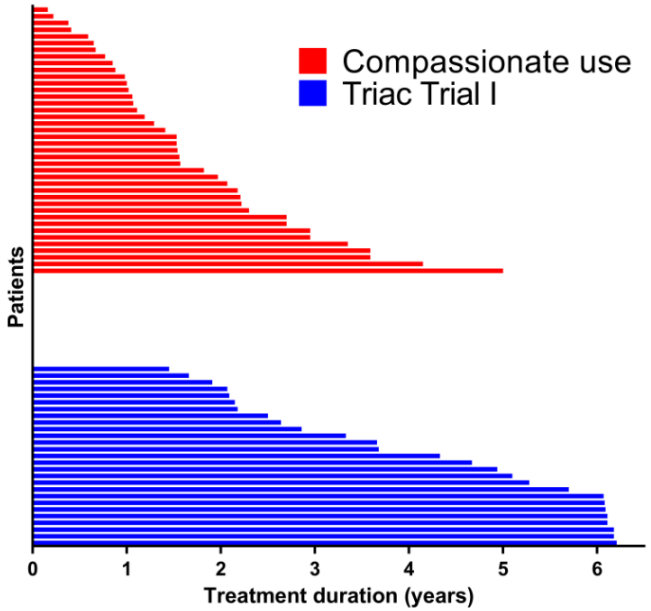
Triac Trial I
N=46

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

New patients
From named patient program
N=40

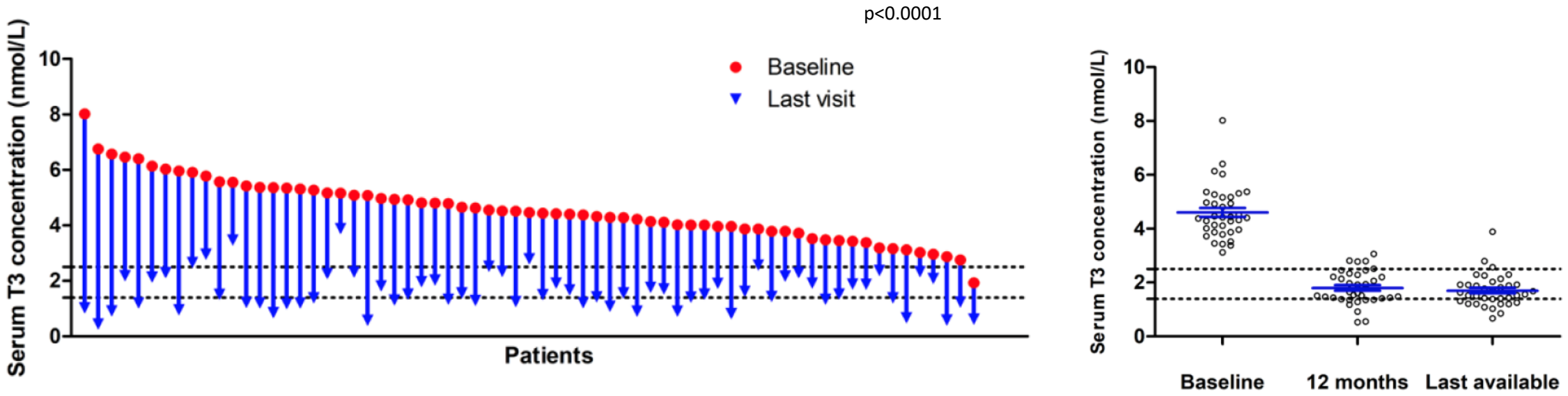
EMC cohort
Published in JCEM
N=67



# 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:** All clinical data available (submission H1 ’23)
**NDA:** Small confirmatory study agreed with FDA (submission mid-’23)

ODD

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

*The voucher may be sold to another sponsor (2021-22 range: $105m-$110m)

# Emcitate regulatory pathway to submissions in EU and US

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

Included in MAA in EU in H1 2023

Included in NDA in US mid 2023 under the Fast Track Designation

To be added post approval when data available

## Triac Trial I

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

## EMC cohort study

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

## Natural history

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

## Controlled study (ReTRIACt)

- Start in Q1 2023
- N= 16
- Pts from named patient/ compassionate use program
- Top line results H1 2023

## 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 intends to submit MAA for Emcitate® to EMA in H1 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

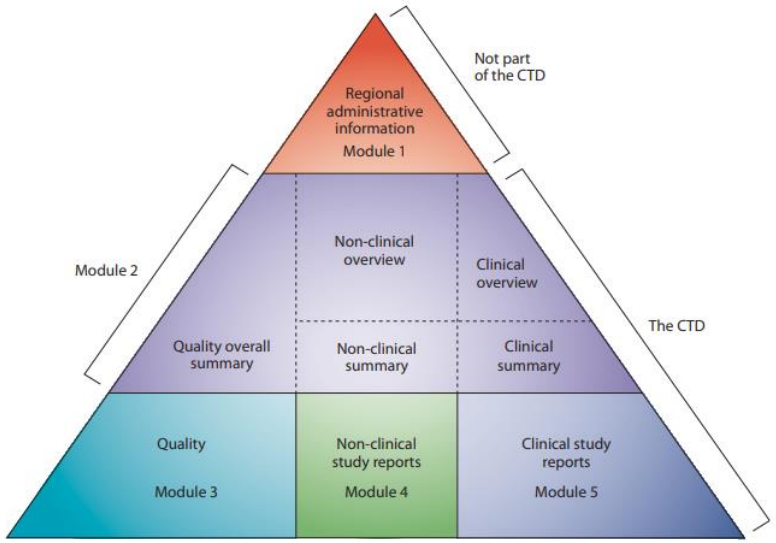
## Egetis intends to submit a marketing authorisation application for Emcitate® to the European Medicines Agency based on existing clinical data

- *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* |
|---|---|---|---|

*Pending stability data

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 2023

- 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 primarily 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 2023** 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.

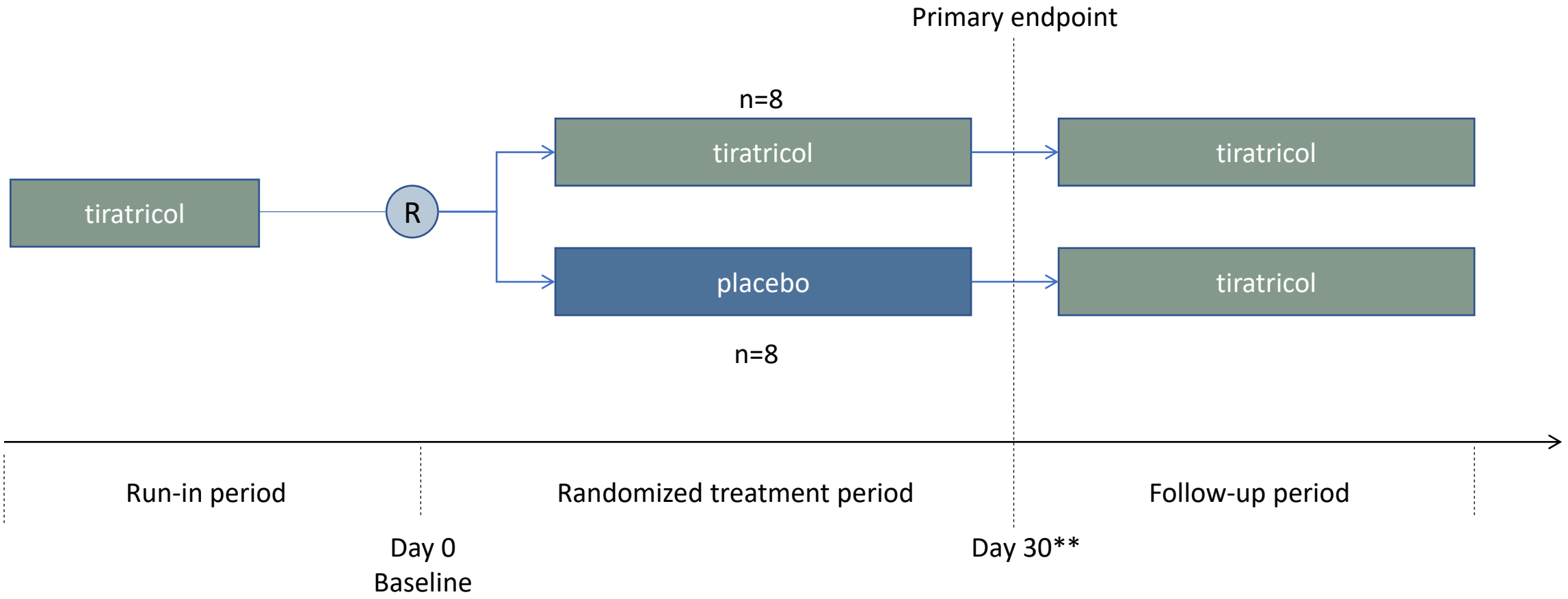
# ReTRIACt: withdrawal of *Emcitate* in males with MCT8 Deficiency

*Randomized placebo-controlled trial needed 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<br>• Change in serum thyroid hormone variables |
| **Description** | • Double-blind, randomized, multicenter placebo-controlled study<br>• Participants with stable maintenance treatment with *Emcitate*<br>• Design agreed with FDA<br>• Clinicaltrials.gov identifier: NCT05579327 |
| **# of patients** | • 16 patients, > 4 years of age<br>• Primary source of patients from NPU program |
| **Timetable** | • Study to start within the next month<br>• Completion expected in H1 2023 |

# Controlled Study (ReTRIACt) – design agreed with FDA

*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

# 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<br>• 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)<br>• Normalization of thyroid hormone function tests and markers of thyrotoxicosis |
| **Description** | • Open label, multi-centre trial in very young children with MCT8 deficiency<br>• International trial with 10 centres in CZ, DE, IT, UK, FR, NL, US<br>• Design discussed and anchored with EMA and FDA<br>• 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<br>• 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<br>• Market approval not dependent on Triac Trial II data |

Note: (1) Gross motor function measure; (2) Bayley Scales of Infant Development

# *Emcitate* milestones and timelines

**2020**
- ✔ US Rare Pediatric Disease Designation
- ✔ Start Triac Trial II

**2021**
- ✔ Fast Track Designation US
- ✔ Data published confirms long-term efficacy & safety of *Emcitate*

**2022**
- ✔ US & EU ODD RTH-β
- ✔ Triac Trial II fully recruited
- ✔ Launch preparations

**2023**
- Start ReTRIACt study for US NDA
- Top line results ReTRIACt
- Filing EU MAA H1 ’23
- Filing US NDA mid ‘23 (Fast Track Designation)
- Launch preparations

**2024**
- EU & US approvals, pricing and launch
- US Priority Review Voucher (upon approval)
- Triac Trial II data

# FDA granted Rare Pediatric Disease designation to Emcitate®

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

## Overview 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 a US Rare Pediatric Disease Priority Review Voucher (PRV) upon approval.
- PRV program recently prolonged until FY 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 2021-22 8 PRVs for rare pediatric diseases have been sold, with individual voucher sale prices 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 |

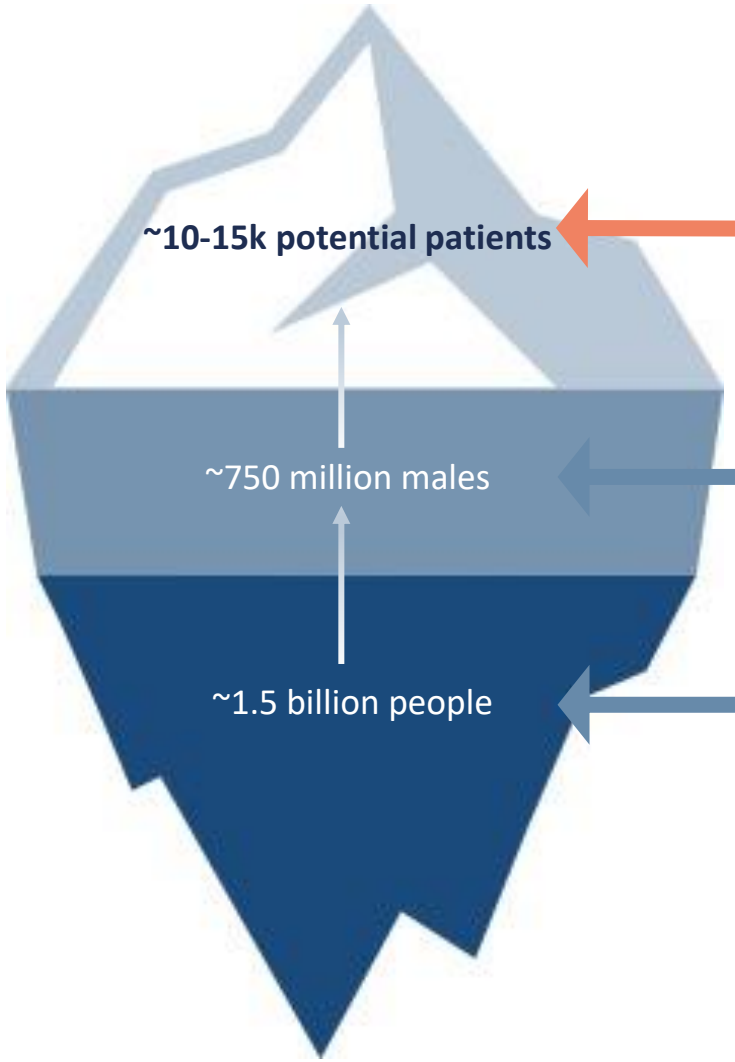
Under the terms of the acquisition of RTT in Q3 2020, 50% of the potential net proceeds from a sale of a PRV related to Emcitate should be paid as an earnout to the sellers of RTT

# 2.

*Emcitate® - Commercial opportunity*

# Estimating 10-15k addressable patients globally

*No approved treatment for MCT8 deficiency*




**MCT8 deficiency prevalence 1:50,000-70,000 males**

Male subset of US, EU and RoW approachable population[1]

US, EU and RoW approachable population[1]

## MCT8 deficiency epidemiology

- At least one new-born diagnosed per 140,000 live births in the Netherlands in past years, corresponding to 1:70,000 males
- Actual number of patients could be higher:
  - Screening study suggests prevalence of 1:50,000 males[2]
  - Once treatment is available, more patients tend to be diagnosed

Note: 1) RoW approachable population, e.g. UK, Australia, Canada, Japan, Switzerland, South Korea and Turkey, 2) Visser et al, Clinical Endocrinology 2012

# 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**

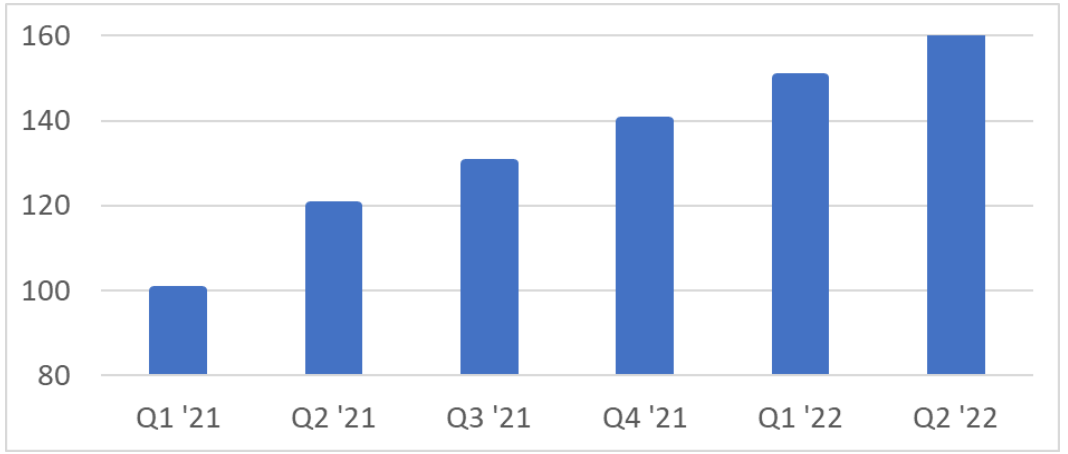
Source: (1) Disease characteristics of MCT8 deficiency: an international, retrospective, multicentre cohort study, Groeneweg et al, The Lancet, 2012; (2) Economic burden of spinal muscular atrophy in the United States: a contemporary assessment, Droege et al, Journal of Medical Economics, 2020;

# 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
- Implemented Expanded Access Program as requested by the FDA - will Simplify Process for Accessing Emcitate
- Emcitate is being supplied on a named patient basis, following individual approval from the national medicines agencies, to
  - more than 160 patients
  - in over 25 countries


**Patients receiving Emcitate in NPU program**



# Commercialization of *Emcitate*

*Disease area conditions provide opportunity for lean commercialization*

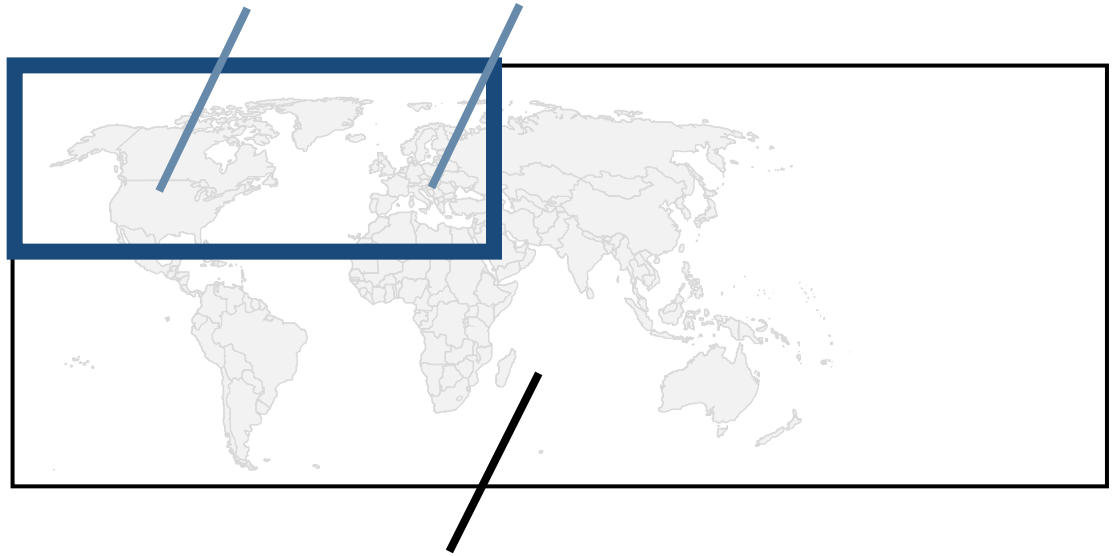
## Favorable conditions for launch success

- **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 inhouse commercial capabilities

- Preparing for **2024 launch** in US and Europe with organization of **40-50** employees at time of launch
- Aiming for rapid access to Emcitate for all **MCT8 deficiency patients**:

***US: 2400* patients*** ***Europe: 5400* patients***



**Plan to commercialize in rest of world through partners**

*Based on prevalence 1:70,000 males

# 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

Anny Bedard, US
President Egetis North America

SAREPTA THERAPEUTICS

Shire

Marianne Berrens-Peijnenburg, NL
Global Head
Medical Affairs

SANOFI GENZYME

Nigel Nicholls, UK
GM for UK & Northern Europe

BIOMARIN

Nadia Georges, CH
Global Head
Market Access & Pricing

SANOFI

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

VERTEX

Peter Verwaijen, NL
Global Head
Marketing & Brand Strategy

ACTELION

John Walsh, US
VP Medical Affairs North America

Biogen

# Focusing on Critical Areas for Launch Success

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

### IDENTIFY PATIENTS

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

### ENSURE ACCESS

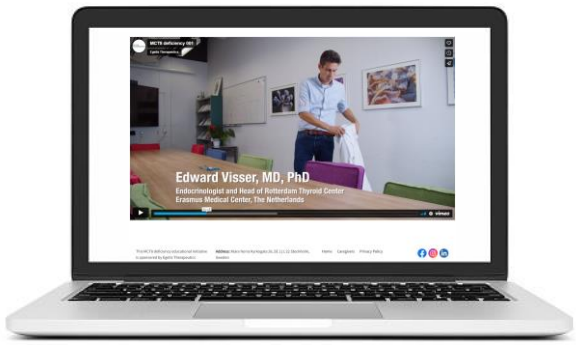
Preparing for broad access to Emcitate as soon as possible after marketing authorization

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

# Enabling patient identification through disease awareness

*MCT8 deficiency awareness and educational activities launched through various channels*

mct8deficiency.com




MCT8 - AHDS Foundation

*Ultra-rare disorder*
Striving for a cure. Empowering families



## DISEASE AWARENESS AND EDUCATION

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

## COLLABORATION WITH PAGs & KOLs

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

## EXHIBIT AT SCIENTIFIC/MEDICAL CONFERENCES

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

## OPTIMUM CHANNEL MIX FOR MAXIMUM REACH

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

# Aiming for broad access 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

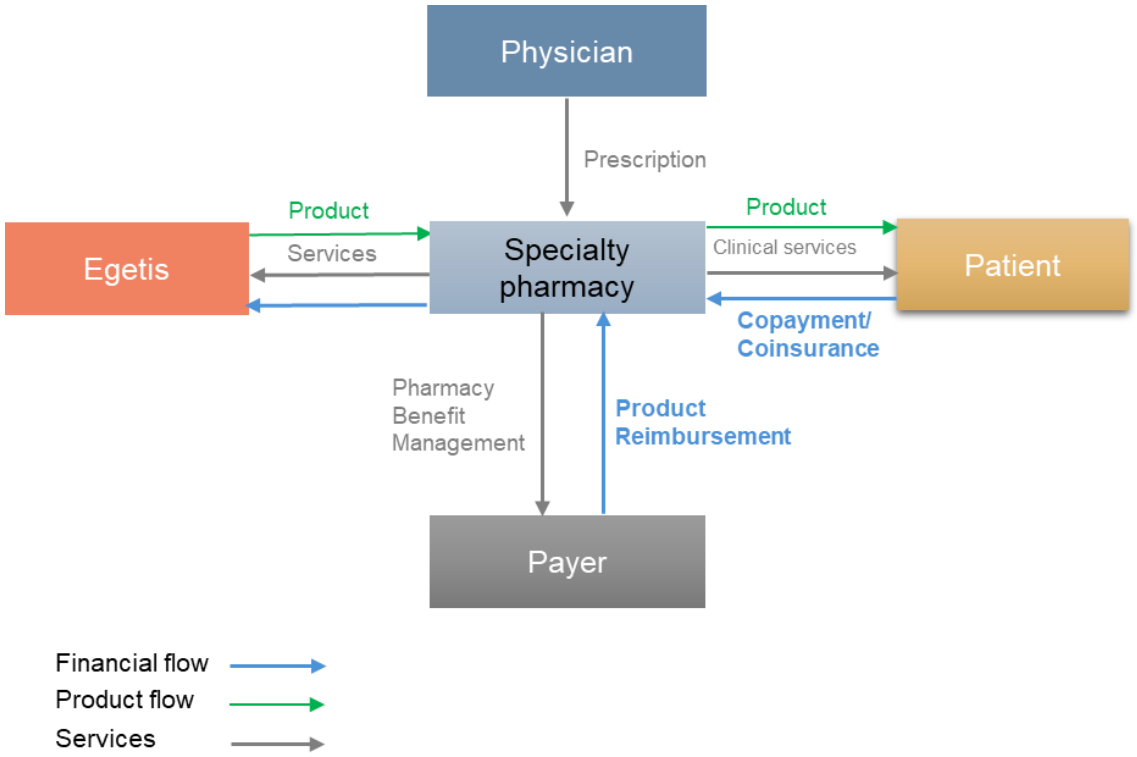
Neuronal ceroid lipofuscinosis type 2 (CLN2) disease is part of a group of rare, progressive, degenerative, neurometabolic disorders called neuronal ceroid lipofuscinoses (NCLs; also known as Batten disease) [1–3]. This rare, autosomal recessive disorder is caused by pathologic variants (mutations) in 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

# Implemented Expanded Access Program as requested by the FDA - will Simplify Process for Accessing Emcitate

- High demand for single patient INDs (Investigational New Drug) resulting in process delays
- FDA Requested Expanded Access Program Transition - Simplify Process for Accessing Emcitate
- Patient Advocacy efforts focused on educating important stakeholders
- Incorporate the patient voice

# 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
  - Aim for no family to pay out of pocket

# 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**

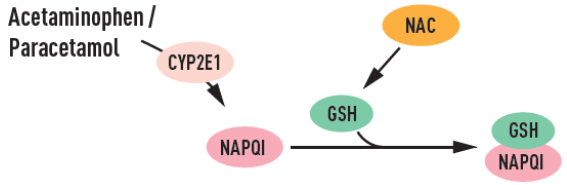
- **Aladote®** aims to become **a new standard of care** for patients with increased risk for liver injury in combination with NAC

# Orphan drug candidate

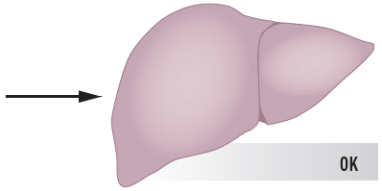
*with clear scientific and mechanistic rationale*

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

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

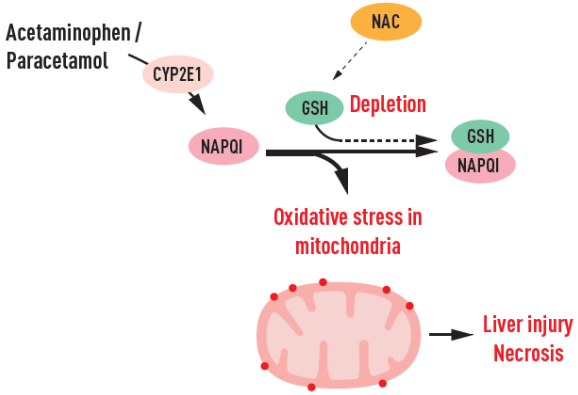


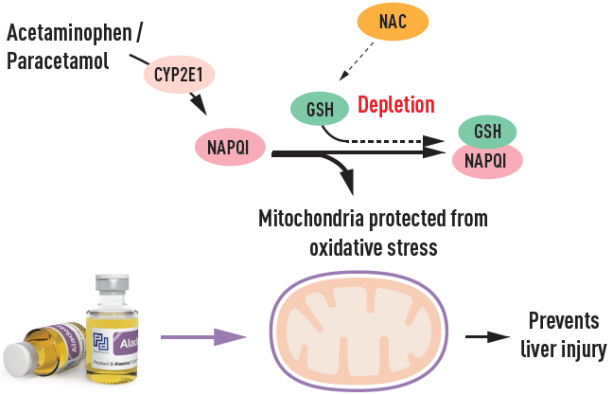

- In most cases NAC effectively prevents liver injury i.e. limited need for Aladote®




## 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 Oxygene 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<br>• 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<br>• 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<br>• 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)<br>• Completed in September 2018 |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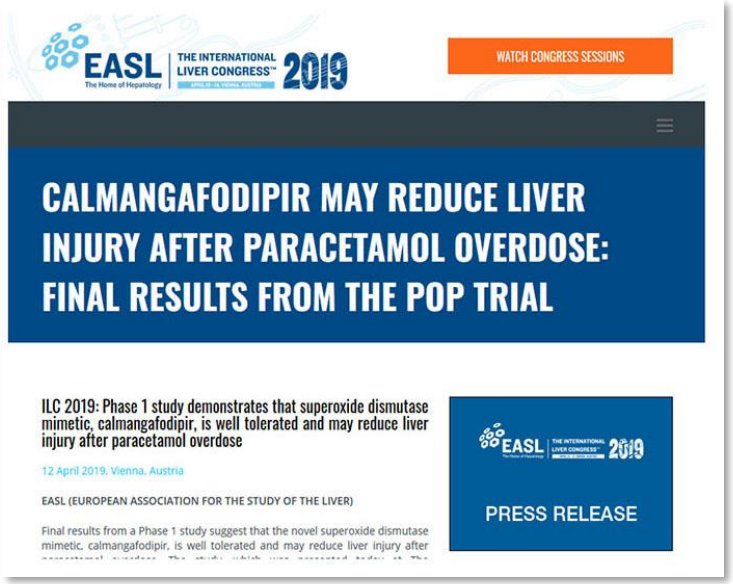
Principal results of a randomised open label exploratory, safety and tolerability study with calmangafodipir in patients treated with a 12 h regimen of N-acetylcysteine for paracetamol overdose (POP trial)

Emma E. Morrison [a], Katherine Oatey [b], Bernadette Gallagher [c], Julia Grahamslaw [c], Rachel O'Brien [c], Polly Black [c], Wilna Oosthuyzen [d], Robert J. Lee [b], Christopher J. Weir [b], Dennis Henriksen [d], James W. Dear [a,*], On behalf of the POP Trial Investigators [1]



# 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%) |

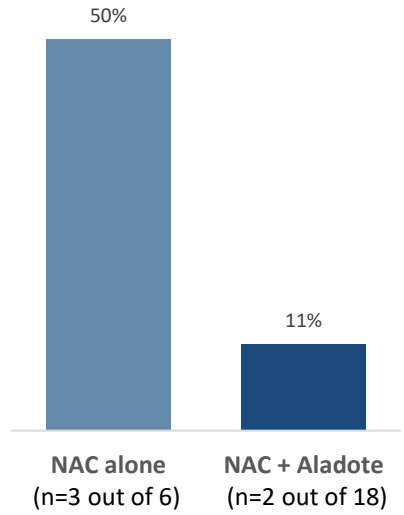
- Met primary endpoint of safety tolerability in the combination of Aladote® and NAC
- No AE or SAE probably or definitely related to Aladote®

## 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

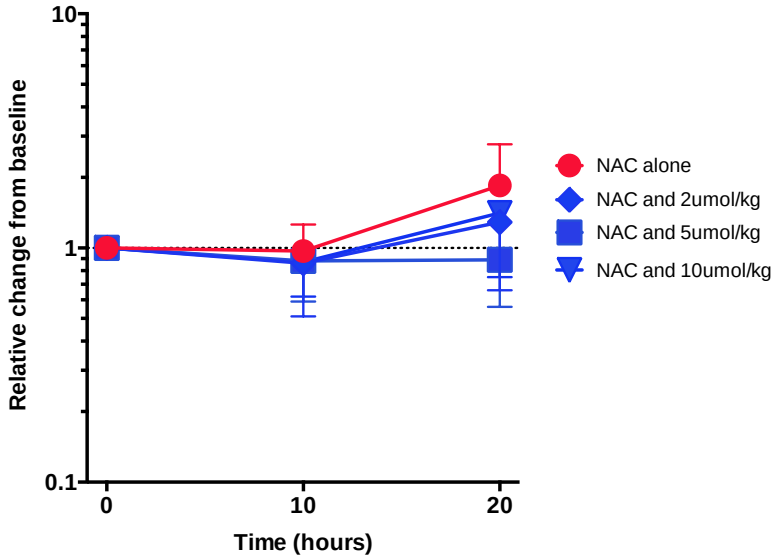
**% of patients needing additional NAC infusions after planned 12h NAC infusion**




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

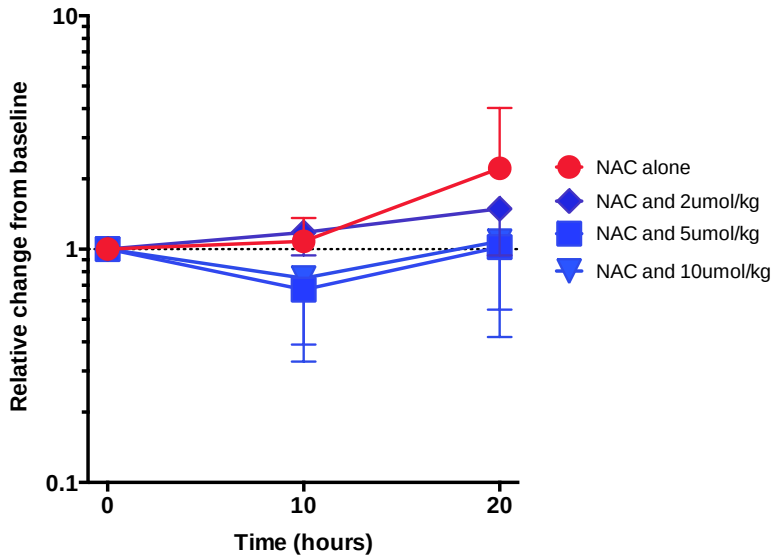
# Aladote® demonstrates consistent results of reduced liver injury as measured by exploratory biomarkers

**K18**



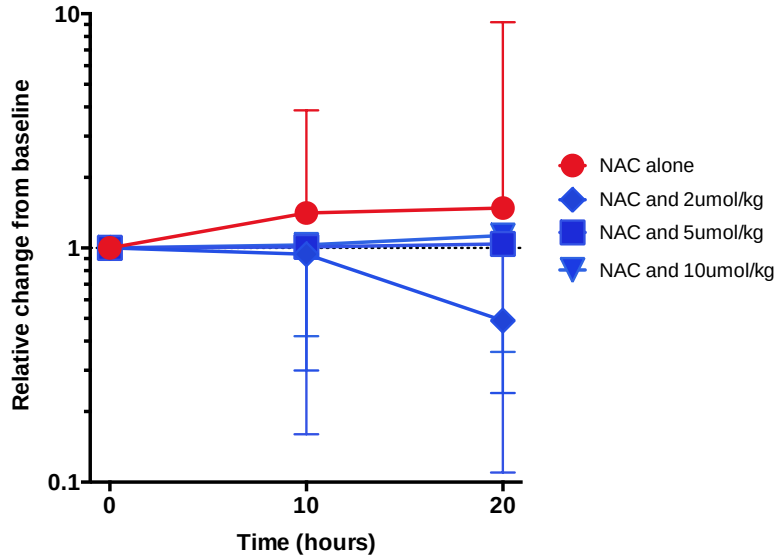

K18 is a measure of cell death and correlate with peak ALT activity during the hospital stay

**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*

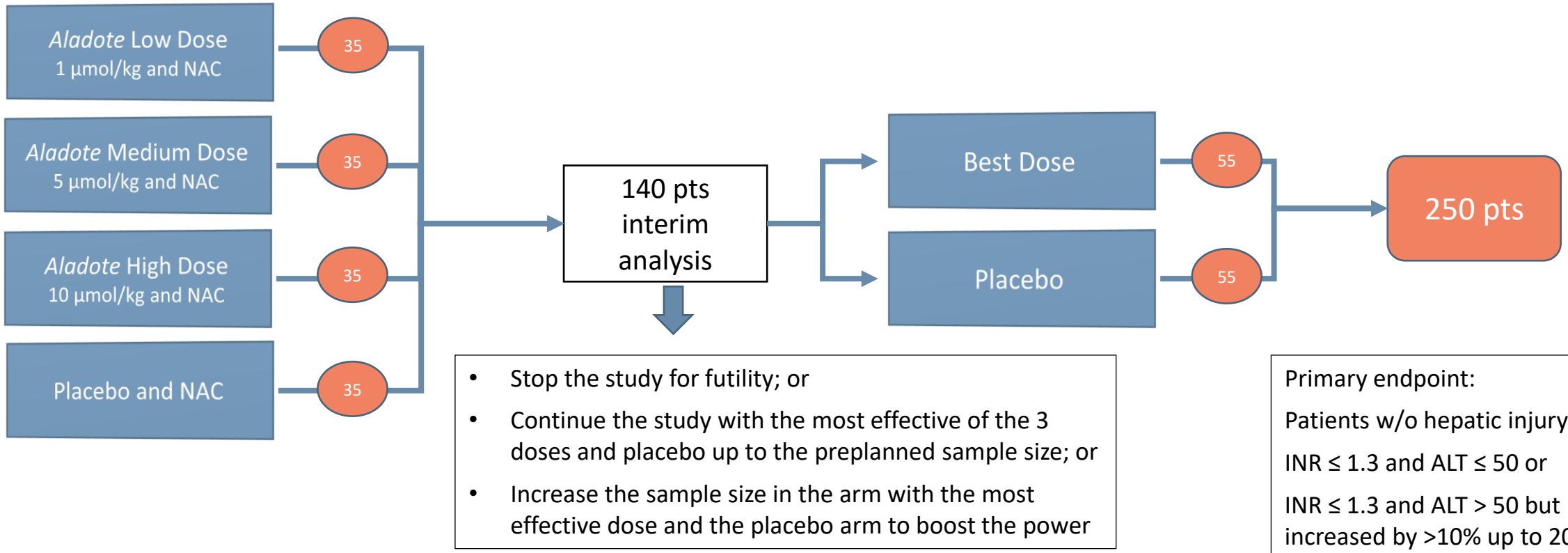
# ALBATROSS: Phase IIb/III study for US/EU regulatory submission

| | |
|---|---|
| **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<br>• Number (%) of patients who need extended NAC treatment after 21 hours<br>• Length of hospital stay<br>• 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

**2023**
- Start pivotal Phase IIb/III study

**2024**
- Interim analysis
- Recruitment completed and topline results

**2025/26**
- 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

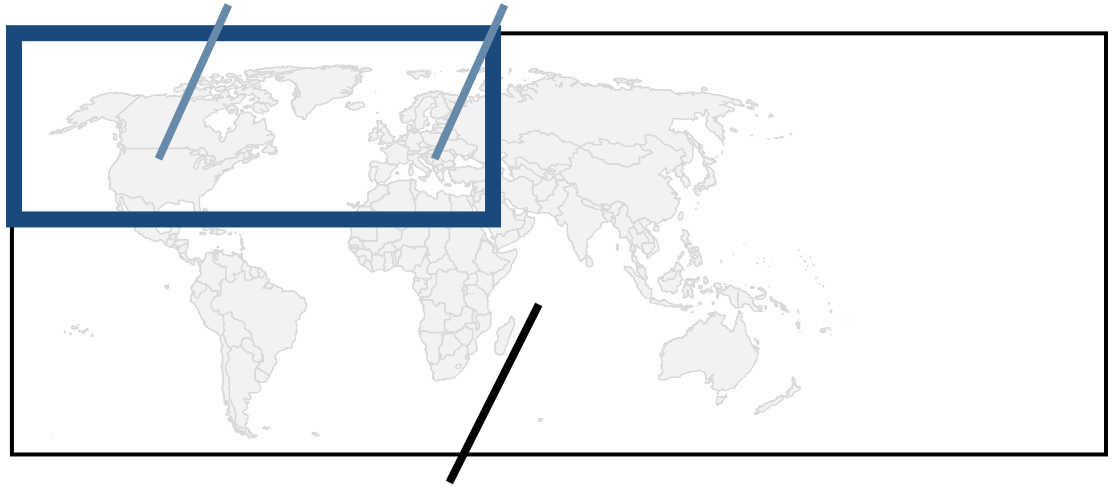
- **Addressing unmet medical need** ✓
- **Leading KOL support** ✓
- Centralized**, focused target groups** of **specialists** eager to improve care ✓
- Treatment choice **highly protocol driven** ✓
- **No competition** ✓

## 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**

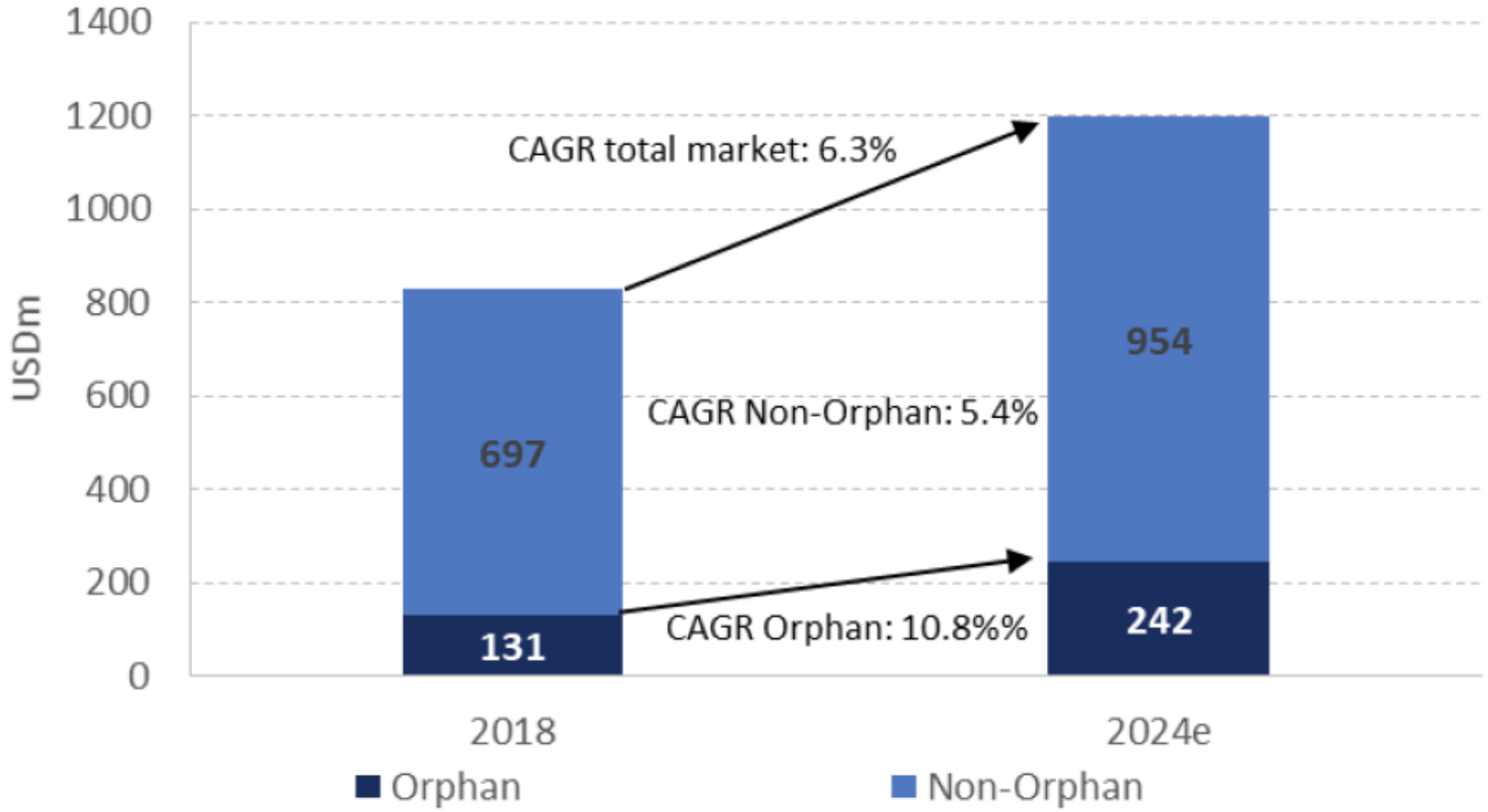
- No or few competitors
- Highly focused target groups
- Premium pricing

**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 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 market in 2024, already passed most of typical drug development risks
  - All clinical data necessary for regulatory approval in EU already at hand – Submission H1 2023
  - A small and short trial reconfirming the effect on biomarker T3 under way to complete the US dossier - Submission mid 2023
- Significant market opportunity with potential for premium orphan drug pricing
  - Estimated 2,400 affected patients in US and 5,400 in Europe
- Eligible for priority review voucher upon US approval, which can be sold for ~100 MUSD

# Two highly promising orphan drug candidates

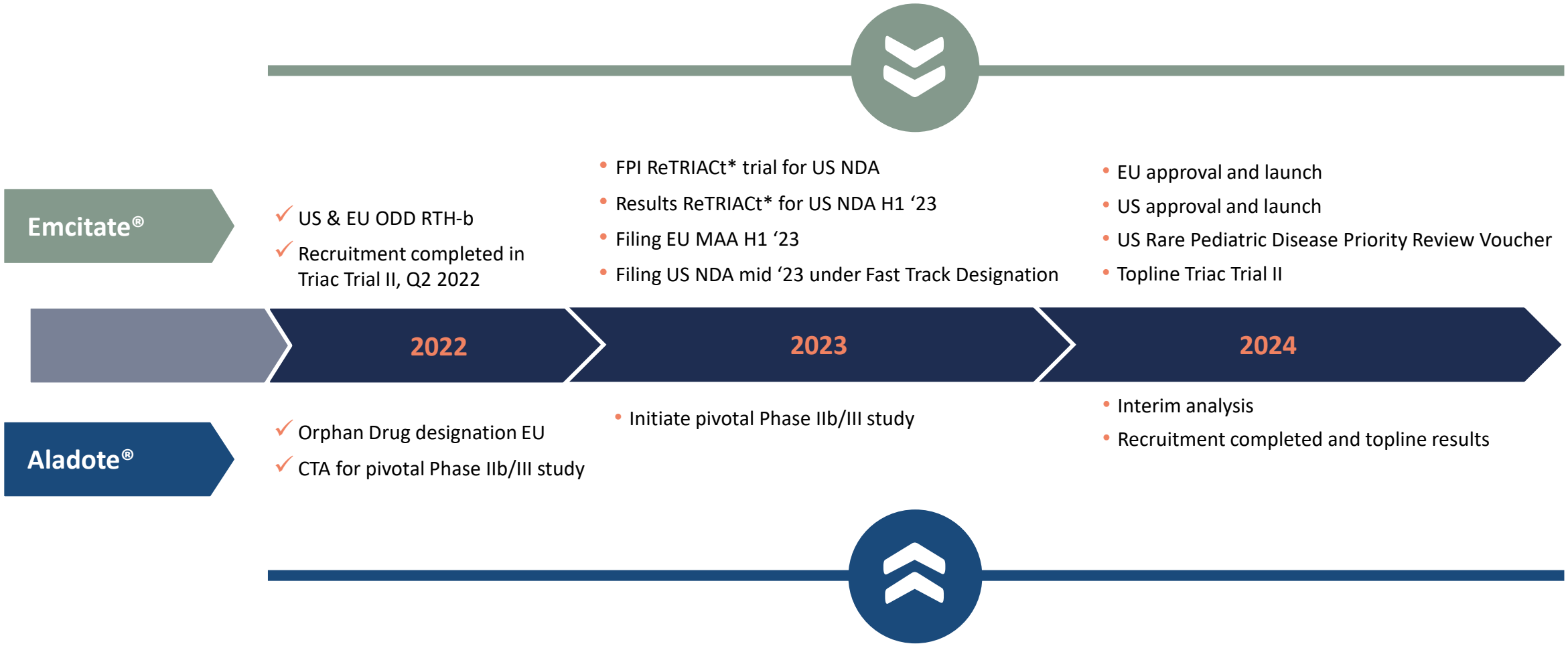
## Emcitate® – *Therapy for MCT8 deficiency*

- MCT8 deficiency affects ~1:70,000 males: high unmet medical need, no available treatment. No competing sponsored products in clinical development
- ODD 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** in **H1 2023**, based on existing clinical data
- **NDA** in **mid 2023**, after conducting a 30 days placebo-controlled study 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 in H1 2024
- More than **160 patients** are being **treated** with Emcitate on a **named patient basis**

## 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, targeting study start in 2023
- No competing products in clinical development

# Upcoming pipeline milestones




* 16 pts randomized 30 day study for US NDA

# 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. Target **MAA/NDA** submissions:
   *Emcitate* **2023** and *Aladote* **2025**
3. Highly attractive **orphan drug segment** with potential **>$1Bn annual sales opportunity**
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

# A.

*Appendix*

# Leadership team with global experience & proven track record

**Nicklas Westerholm**

*CEO*

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

**Yilmaz Mahshid, PhD**

*CFO*

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

**Henrik Krook, PhD**

*VP Commercial Operations*

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

**Karl Hård, PhD**

*VP IR, Communications & Business Development*

- Joined 2022
- Redx Pharma, Kiadis, AstraZeneca

**Anny Bedard**

*President Egetis North America*

- Joined 2022
- Commercial leadership roles at Shire and Sarepta

**Kristina Sjöblom Nygren, MD**

*CMO*

- Joined 2020
- 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
- AstraZeneca 13 years
- Late stage development expertise from FORXIGA, MOVANTIK, ONGLYZA, BRILINTA & QTERN

# Board of directors

## Thomas Lönngren

*Chair of the board*

- Board member since 2021
- MSc in social and regulatory pharmacy and a degree in Pharmacy, University of Uppsala.
- Previously Executive Director of the European Medicines Agency
- Board member at Compass Pathways and NDA Group

## Peder Walberg

*Board member*

- Board member since 2020
- Founder and CEO of Rare Thyroid Therapeutics
- MD and BSc in international economy and business administration, Uppsala University
- Other assignments: Board Member of Immedica
- Previous assignments: Founder and CEO, Medical Need, Head of Business Development and Strategy, Swedish Orphan and SOBI. BoD of Wilson Therapeutics and identified Decuprate for treatment of Wilson disease

## Gunilla Osswald

*Board member*

- Board member since 2017
- PhD in biopharmacy and pharmacokinetics
- Other assignments: CEO BioArctic AB

## Elisabeth Svanberg

*Board member*

- Board member since 2017
- MD, PhD, Assoc Professor in surgery
- Other assignments: Chief Development Officer Ixaltis SA. Board member Leo Pharma, Amolyt Pharma and Galapagos

## Mats Blom

*Board member*

- Board member since 2021
- BA, Business Administration and Economics, University of Lund and MBA, IESE University of Navarra
- Other assignments: CFO NorthSea Therapeutics and Board member of Hansa Biopharma and Auris Medical

# Share Register and Market Cap

## 10 largest shareholders

| Name | Capital | Votes | Num. of shares | Verified |
|---|---|---|---|---|
| Peder Walberg | 15.74% | 15.74% | 33 776 221 | 2022-09-28 |
| Peter Lindell | 10.40% | 10.40% | 22 320 000 | 2022-09-28 |
| Fjärde AP-fonden | 8.67% | 8.67% | 18 604 690 | 2022-09-28 |
| Avla Holding AB | 8.23% | 8.23% | 17 668 330 | 2022-09-28 |
| Flerie Invest AB | 6.19% | 6.19% | 13 280 571 | 2022-09-28 |
| RegulaPharm AB | 4.91% | 4.91% | 10 531 660 | 2022-09-28 |
| Linc AB | 3.00% | 3.00% | 6 432 021 | 2022-09-28 |
| Avanza Pension | 2.53% | 2.53% | 5 418 733 | 2022-09-28 |
| Unionen | 1.99% | 1.99% | 4 275 833 | 2022-09-28 |
| Carl Rosvall | 1.64% | 1.64% | 3 520 287 | 2022-09-28 |
| **Total 10** | **63.30%** | **63.30%** | **135 828 346** | |
| Total number of owners | 6,446 | | | 2022-09-30 |
| Total number of shares | 214,589,128 | | | 2022-09-30 |

- Cash position: SEK 190 M (~EUR 18M)*
- Number of outstanding shares: 214.6M
- MCap: ~SEK 1,5 billion**
- Listing venue: Nasdaq Stockholm Main Market

*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 Sept 30, 2022 (Q3 2022 report); ** January 4, 2023*

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# 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;

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# Thank you!

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