



Corporate presentation

May 2025

Egetis has launched Emcitate® in the first country, Germany, on May 1, 2025
NDA submission planned for 2025

Disclaimer

Tiratricol is an investigational drug and has not been approved by the U.S. Food and Drug Administration (FDA). Its safety and efficacy have not been established in the U.S.

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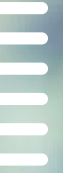
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WE CARE
FOR THE RARE



1.

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

Egetis: building an orphan drug commercial stage company



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

A strong regulatory status

ODD

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

Fast
track

Fast track designation - FDA

PRV

Rare pediatric disease designation - FDA
Priority Review Voucher upon approval

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

ALEXION

Sobi
Pioneer in Rare Diseases

WILSON
THERAPEUTICS

BIOMARIN

VERTEX

Biogen

Medical Need

NOVARTIS

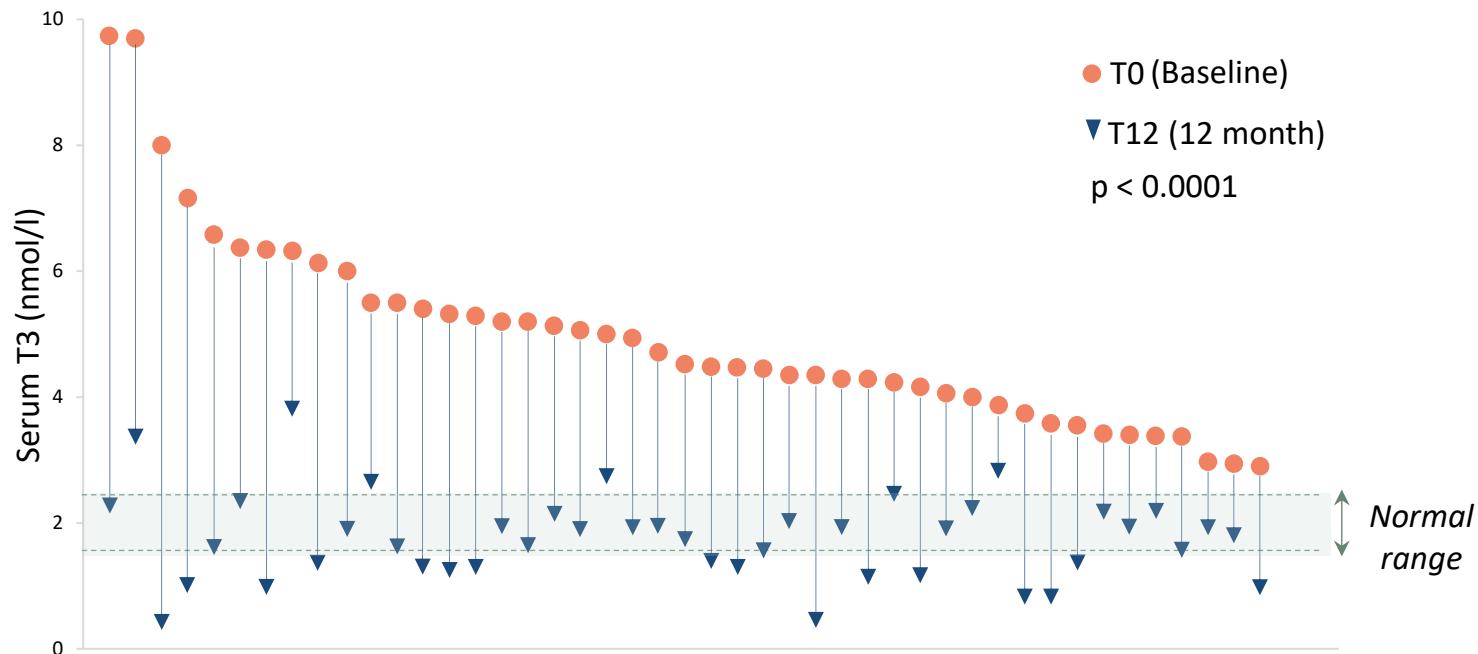
AstraZeneca

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



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



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

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

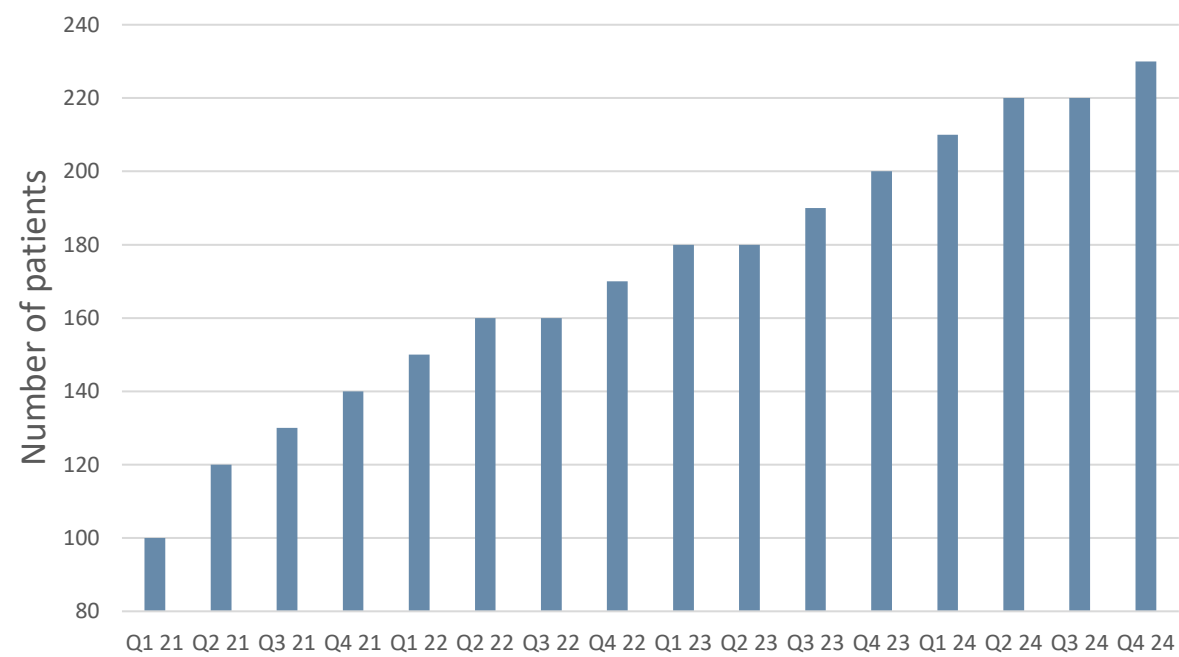
Key demonstrated clinical results

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

Emcitate already supplied globally in managed access programs

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

Patients Receiving *Emcitate* in Managed Access Programs



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

MCT8 deficiency key features

Estimated incidence: 1 case per 70k male

Median onset of symptoms: 4 months

Median age of diagnosis: 10 months

Patients surviving into adulthood: 70%

Severe intellectual disability: 100%

Ability to sit independently: 8%

Hypotonia, hypertonia

& persistence of primitive reflexes: 90%

Severe underweight: 75%

Cardiac arrhythmias (PAC): 76%

Median life expectancy: 35 years

Patients dying in childhood: ~30%

Main cause of mortality: Sudden cardiac death

Life long 24-hour care: 100%

Emcitate/tiratricol regulatory pathway in EU/US

Robust data set in an ultra rare genetic disease



Triac Trial I	EMC cohort study	Natural history	Triac Trial II	Survival study		ReTRIACt Trial
				Abstract at ETA (EMA)	Full data set (FDA)	
N=46	N=67	N=151	N=22	N=228	N>228	N=16
<ul style="list-style-type: none">Completed 2018 (Groeneweg, 2019)Open-label, international, multi-centre study	<ul style="list-style-type: none">Completed 2021 (van Geest, 2022)N= 27 from Triac Trial I & N= 40 new pts from managed access program	<ul style="list-style-type: none">Retrospective data, 2003 to 2019 (Groeneweg, 2020)	<ul style="list-style-type: none">Open-label, international, multi-centre studyFocus on neurocognition; did not meet its primary endpoints96 weeks safety data in young patients	<ul style="list-style-type: none">International real-world cohort studyComparing treated vs untreated patients on survivalTreatment with tiratricol (Emcitate®) is associated with a 3x lower risk of mortality.	<ul style="list-style-type: none">N= 16Placebo-controlled withdrawal studyOngoing	

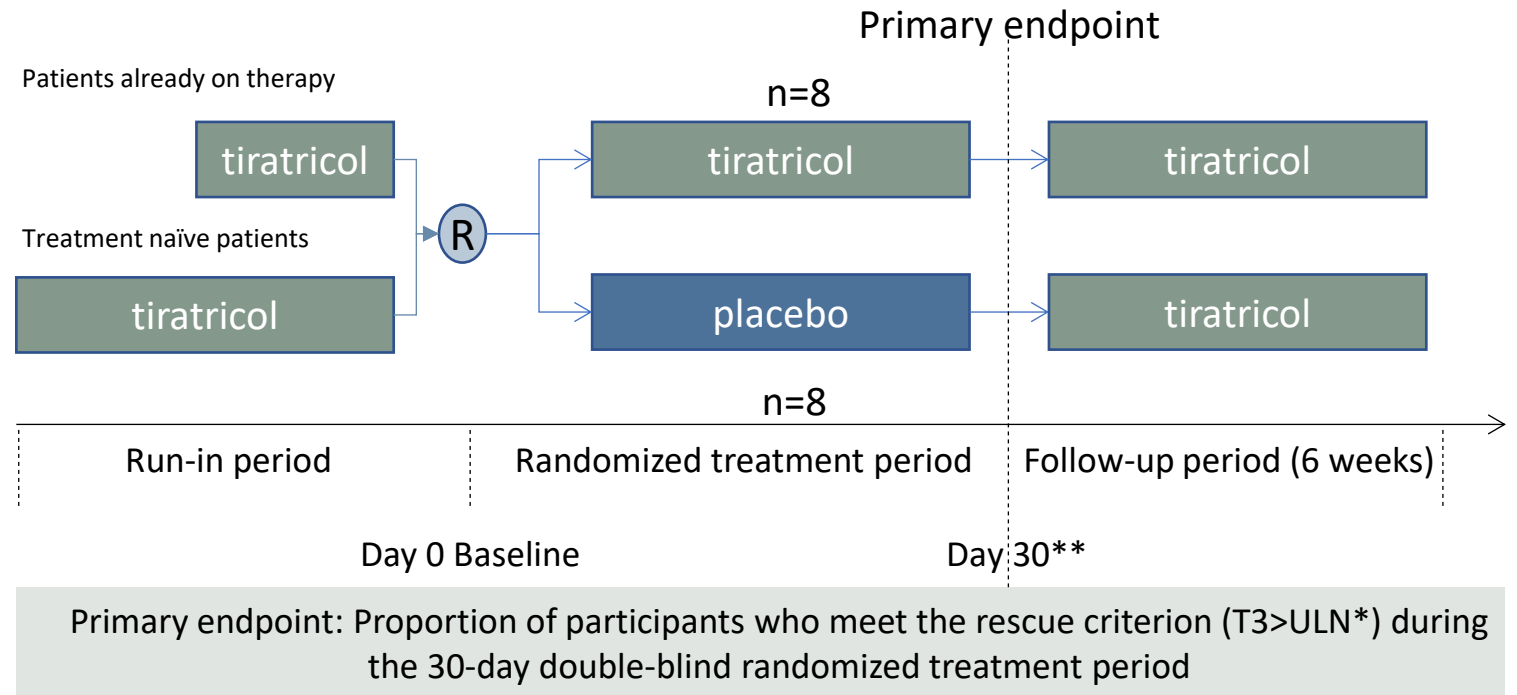
Data included in EMA MAA – EC approval Feb 2025

Data to be included in FDA NDA

Design of the ReTRIACt clinical trial (status as of April 30, 2025)

Requested by the FDA

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



- 12 patients have completed the randomized phase, 1 patient is in the randomized phase and 1 patient is in the run-in period. Two patients are planned for screening and additional patients are being evaluated for screening.
- 7 sites currently open, including the sites in Georgia, North Carolina, Texas and Florida.
- Recruitment will continue until at least 16 patients have completed the randomized phase.

Egetis will update the market as soon as recruitment has been completed, and subsequently when top-line results are expected. NDA filing is planned for 2025.

* ULN: Upper Limit of Normal

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

A phased EU launch through in-house commercial organization started in Germany May 1

Pricing & Reimbursement (P&R) strategy execution in 2 waves, starting with EU4

Wave 1

France, Germany, Italy, Spain



Wave 2

Phased on a country-by-country approach

Rest of Europe



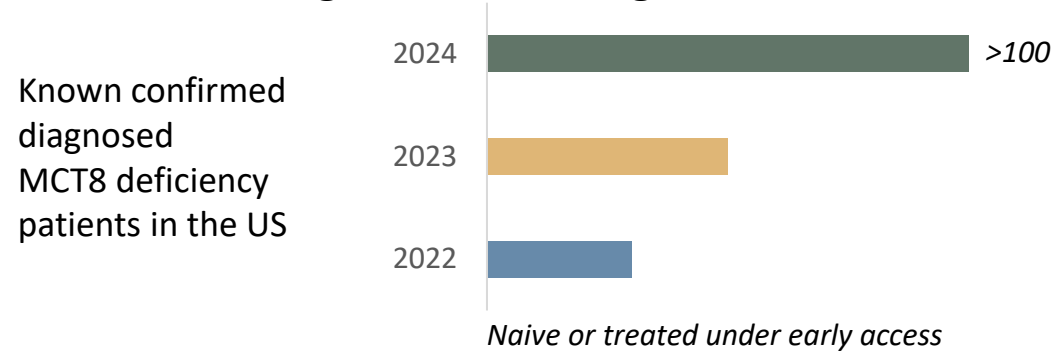
Pricing &
Reimbursement
processes

Deliver the *Emcitate* clinical and economic value proposition in P&R processes, outlining:

- MCT8 deficiency and its rarity
 - Summarizing available literature
- High burden of MCT8 deficiency
 - Confirmed by Egetis sponsored Caregiver study
- Significant unmet medical need
 - Emcitate the first & only approved treatment
- Benefit of treatment
 - Supported by publications & ETA guidelines

US commercial preparedness activities under way

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



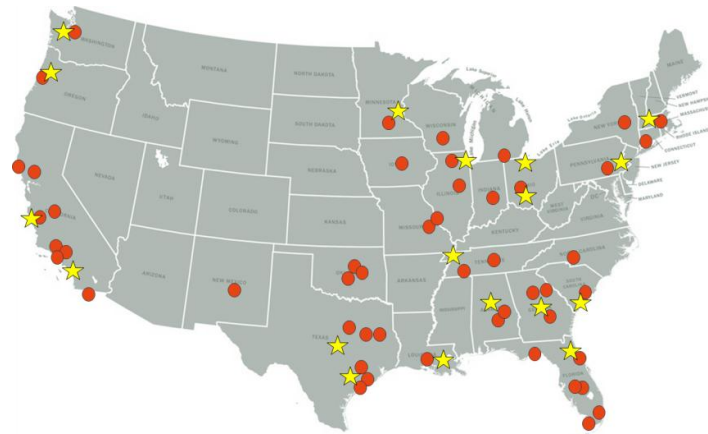
Expanded Access Program, requested by FDA, is a significant asset to both patients and Egetis launch readiness

- Providing early and sustainable access to therapy
- Physicians gain experience of tiratricol
- Opportunity to generate real world data to support regulatory and payer interactions

Cover major US centers through pre-commercial initiatives

● Hospitals with known MCT8 deficiency patients

★ Tiratricol Expanded Access Program sites, active or in process



Patient-centric implementation

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

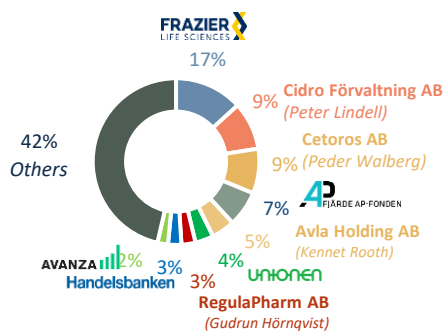
Disclaimer: Tiratricol is an investigational drug and has not been approved by the US Food and Drug Administration (FDA). Its safety and efficacy have not been established for the US market.

Strong financial foundation for strategic execution

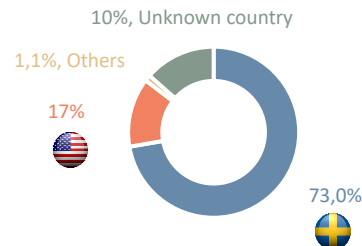
Solid cash position

- **Cash position April 30, 2025:** SEK 273 million
- **Number of outstanding shares:** 359,238,126
- **Market Cap:** ~SEK 1.4 billion*
- **Listing venue:** Nasdaq Stockholm, Main Market
- **Ticker:** EGTX

% capital by shareholders



% capital by geographical distribution



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

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

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

Note: * May 12, 2025

Upcoming value enhancing key milestones 2025-2026



Emcite®

2025-2026

MCT8
deficiency

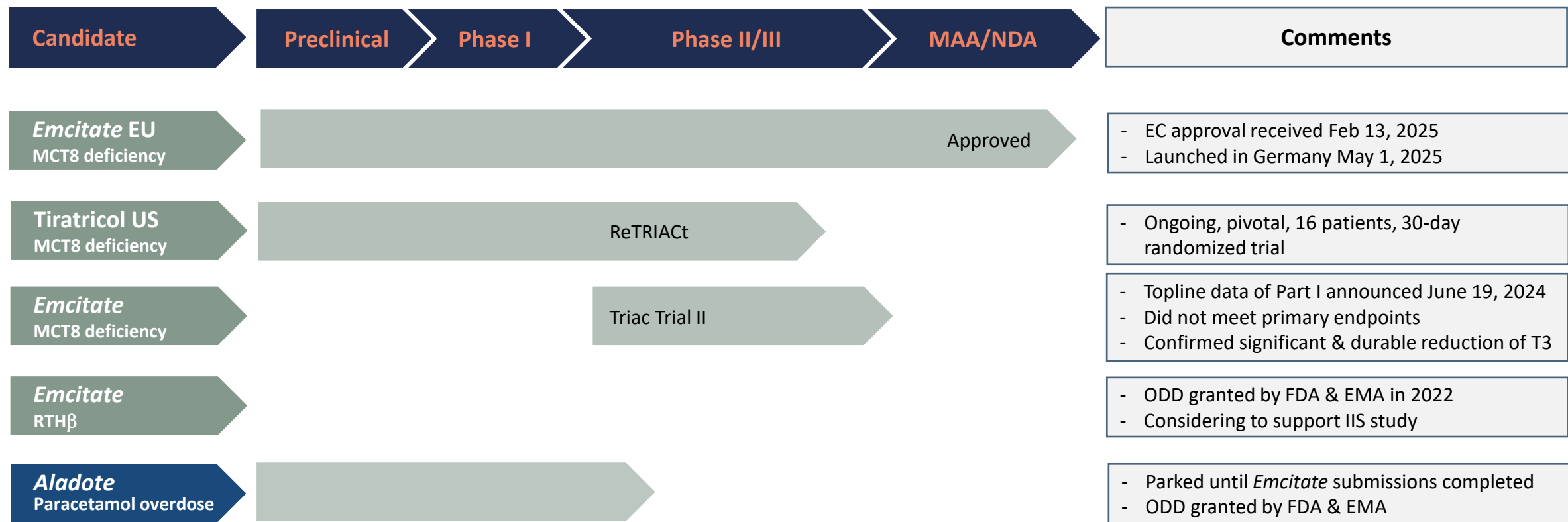
- EU launch, in the first country, Germany, May 1, 2025
- Topline results ReTRIACt for US NDA
- Submission of US NDA – priority review
- Middle East, North Africa & Turkey partnership/s
- Japan – Development plan agreed with PMDA
- US Patent granted - Processes and compounds
- US approval and launch
- US Rare Pediatric Disease Priority Review Voucher

RTH-beta

- Potential initiation of Investigator Initiated Study - Egetis Industry collaborator

Pipeline overview

Emcitate – European Commission approval Feb 13, 2025



EC: European Commission; IIS: Investigator Initiated Study; ODD: Orphan Drug Designation;
RTHβ: Resistance to Thyroid Hormone beta; T3: thyroid hormone T3 (triiodothyronine)

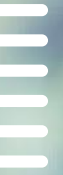
Building a sustainable orphan drug company

- Successfully develop *Emcitate* for EU & US approvals in 2025/26 and potentially *Aladote* post 2026
- Commercialize *Emcitate* and *Aladote* through an inhouse organization in Europe/ North America and partnerships in RoW
- Realize the full potential of our products via life-cycle management
- Ensure fast and broad access to our products for the benefit of patients worldwide
- Identify further assets that address the significant unmet medical need for patients with rare diseases
- Provide an open culture that encourages Collaboration, Courage & Commitment
- Egetis financial objective is to create increased value for shareholders in the long term

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

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





2.a

Overview of MCT8 deficiency

MCT8 deficiency results in dysfunctional thyroid hormone trafficking

MCT8 deficiency has two co-manifestations

New Research Sheds Light on Thyroid Hormone Transport

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

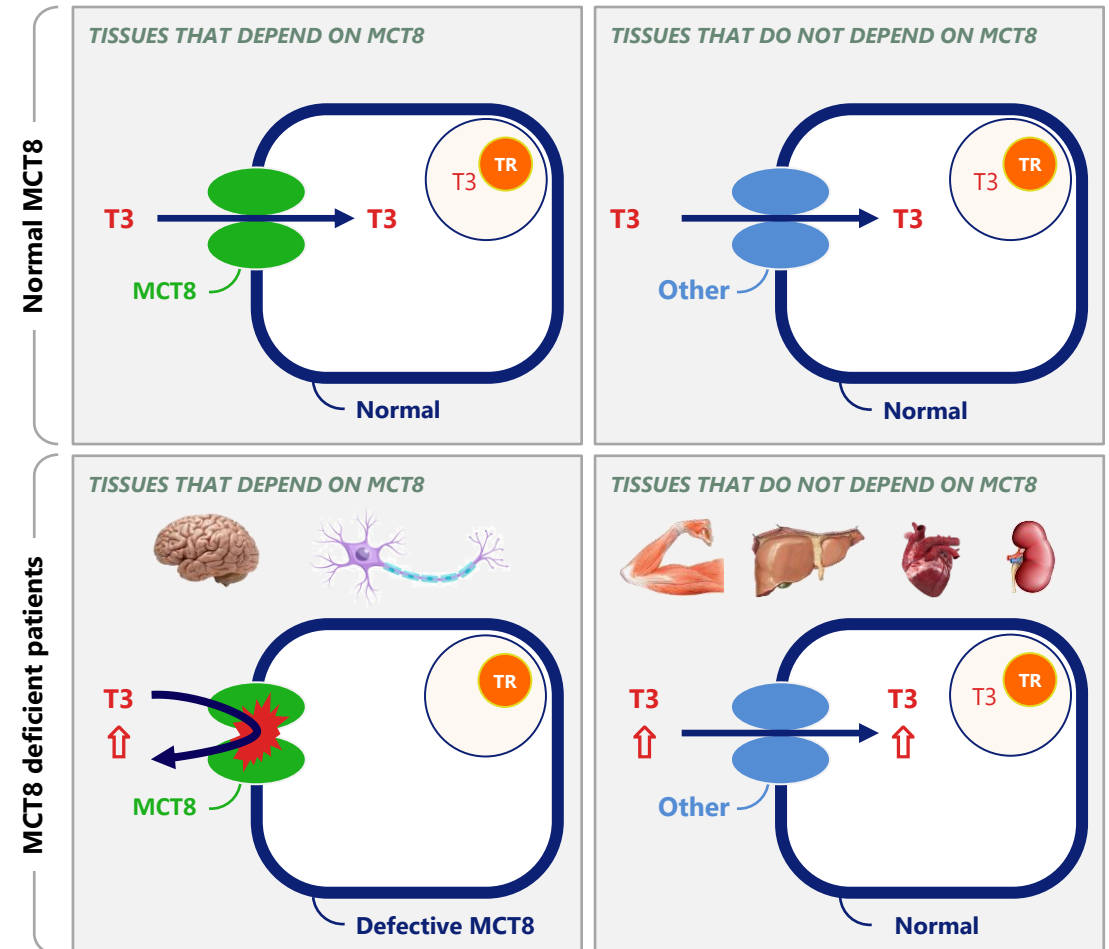
MCT8 Plays a Key Role in Neurocognitive Development

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

And Causes Many Additional Symptoms



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

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



MCT8 deficiency: a detrimental condition with significant unmet medical need



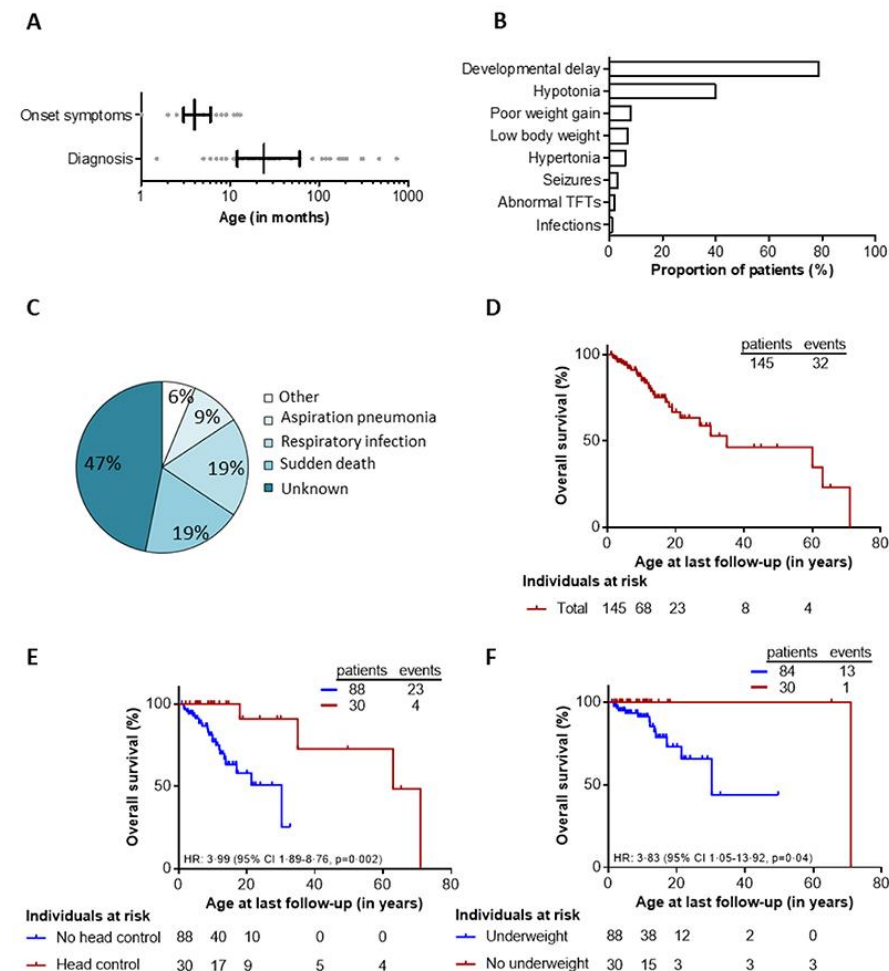
What is MCT8 deficiency?	What does it mean?	What are the challenges?	How do you manage the disease?	Quick facts from natural history ²																										
<ul style="list-style-type: none">Genetic X-linked disorderImpaired thyroid hormone trafficking across cellular membranesMCT8 is a key thyroid hormone transporter in the bodyPrevalence 1:70,000 males <div></div> <p>Patients with MCT8 Deficiency¹⁾</p>	<ul style="list-style-type: none">Non-functional MCT8 protein: T3 cannot cross blood-brain-barrierLow amounts of thyroid hormone in the brain & CNSDisrupted feedback loop results in a compensatory increase in circulating thyroid hormone <div></div> <ul style="list-style-type: none">Simultaneous too high & too low thyroid hormone in different tissues	<ul style="list-style-type: none">Patients appear normal at birthInitial symptoms within the first months of lifeSevere intellectual disabilityMost patients never able to sit or walk; limited ability to communicateLife-long morbidity: agitation, CV symptoms, wasting & impaired life expectancy <div></div> <ul style="list-style-type: none">Heavily dependent on caregivers resulting in very high disease burden	<ul style="list-style-type: none">No available therapyEasy diagnosis once considered with readily available, low-cost lab-testLarge proportion of patients remain undiagnosed with significant delay to diagnosis <div></div> <ul style="list-style-type: none">Significant unmet medical need: humanitarian, health economic, societal	<table><tr><td>Median onset of symptoms:</td><td>4 months</td></tr><tr><td>Median age of diagnosis:</td><td>10 months</td></tr><tr><td></td><td>(prior to 2017: 24 months)</td></tr><tr><td>Patients surviving into adulthood:</td><td>70%</td></tr><tr><td>Severe intellectual disability:</td><td>100%</td></tr><tr><td>Ability to sit independently:</td><td>8%</td></tr><tr><td>Hypotonia, hypertonia & persistence of primitive reflexes:</td><td>90%</td></tr><tr><td>Severe underweight:</td><td>75%</td></tr><tr><td>Cardiac arrhythmias (PAC):</td><td>76%</td></tr><tr><td>Median life expectancy:</td><td>35 years</td></tr><tr><td>Patients dying in childhood:</td><td>~30%</td></tr><tr><td>Main cause of mortality:</td><td>Sudden cardiac death</td></tr><tr><td>Life long 24-hour care:</td><td>100%</td></tr></table>	Median onset of symptoms:	4 months	Median age of diagnosis:	10 months		(prior to 2017: 24 months)	Patients surviving into adulthood:	70%	Severe intellectual disability:	100%	Ability to sit independently:	8%	Hypotonia, hypertonia & persistence of primitive reflexes:	90%	Severe underweight:	75%	Cardiac arrhythmias (PAC):	76%	Median life expectancy:	35 years	Patients dying in childhood:	~30%	Main cause of mortality:	Sudden cardiac death	Life long 24-hour care:	100%
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Note: 1) Picture from Schwarz et al; Clin Endocrinol & Met 2007; 2) Groeneweg et al, Lancet Diabetes & Endocrinology, 2020

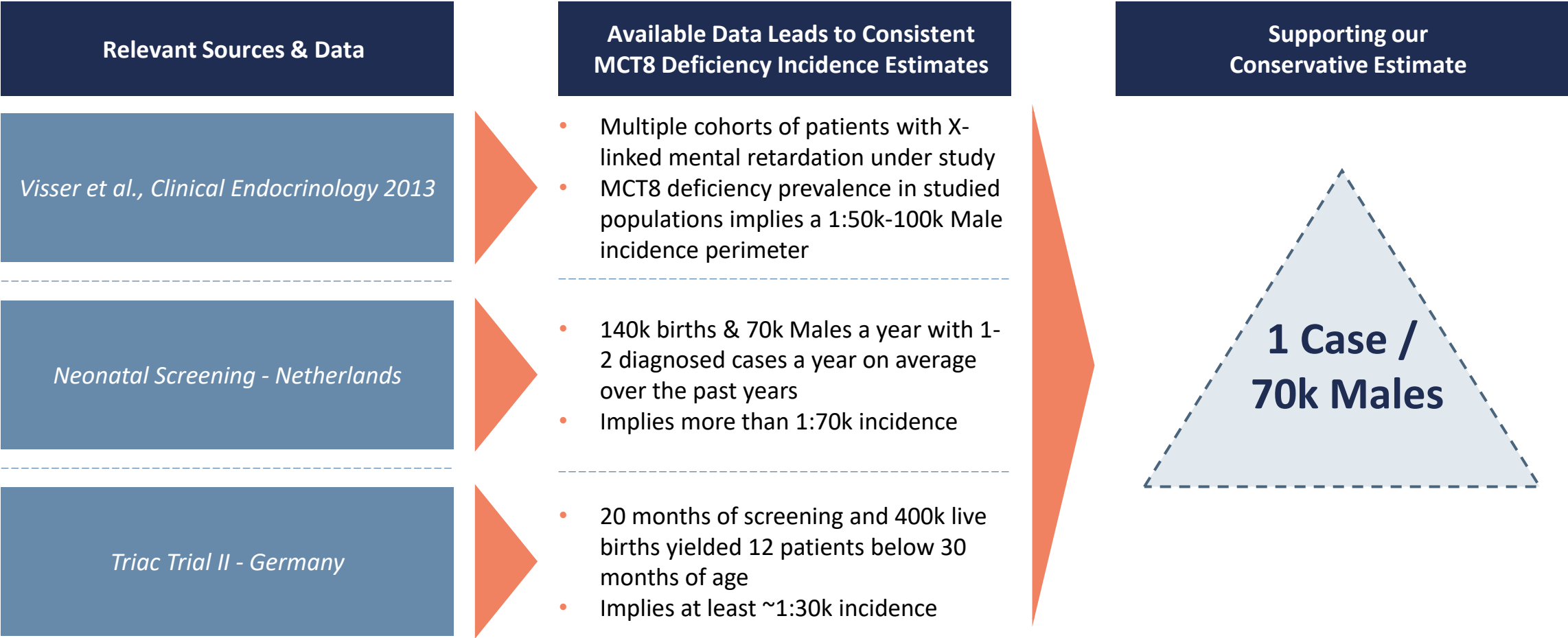
Natural history study revealed poor survival with a high prevalence of treatable underlying risk factors

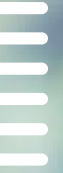
An international, retrospective, multicentre cohort study from 2014-2020 in 151 patients

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



Multiple sources lead to consistent MCT8 deficiency incidence estimates





2.b

Clinical experience with Emcitate

Emcitate® Overview

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



Clinical

- **Triac Trial I (Phase IIb)** completed with significant & clinically relevant effects on **T3 levels** and **chronic thyrotoxicosis**
- **Erasmus Medical Center cohort study confirms long-term efficacy and safety for up to 6 years**
- **Triac Trial II:** Results announced June 2024. Did not meet primary neurocognitive endpoints. Confirmed the significant and durable reduction of T3 levels in all patient
- New study reports tiratricol (Emcitate®) treatment in patients with MCT8 deficiency is associated with **survival benefits**

Regulatory

- Orphan drug designation in EU & US, US Rare Pediatric Disease Designation - **eligible for Priority Review Voucher**
- **Fast track designation** granted by FDA
- **EU approval received on February 13, 2025. Launched in Germany May 1, 2025**
- For the US **NDA submission** a 30-day, placebo-controlled study in at least 16 evaluable patients is being conducted to verify the results on T3 levels seen in previous clinical trials and publications

Commercial

- European Thyroid Association **recommends tiratricol as long-term therapy** for all patients with MCT8 deficiency
- 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 **230 patients** are being treated with *Emcitate* in managed access programs
- Expected **market exclusivity** is **10 years in EU (ODD), 7 years in US (ODD)**

Orphan drug candidate

with clear scientific and mechanistic rationale and established safety profile

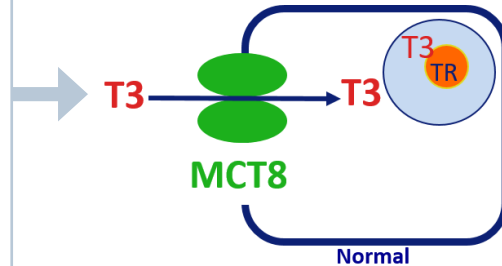


Difference normal MCT8 and deficiency of MCT8

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

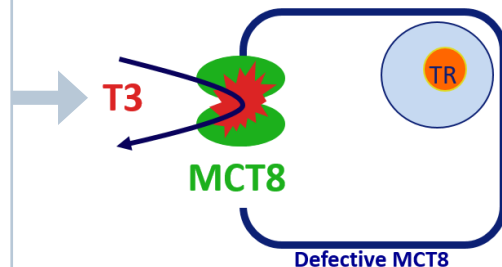
Normal MCT8 ✓

- 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

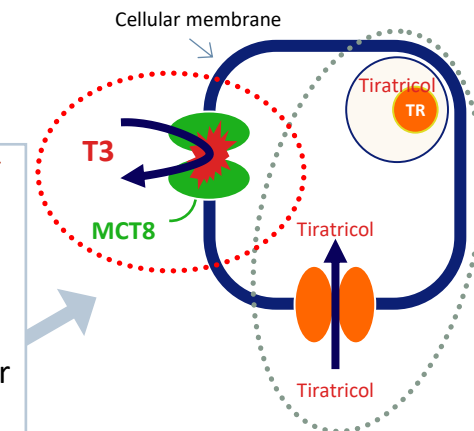


Emcitate (tiratricol) – Addressing MCT8 deficiency

- 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 ✗



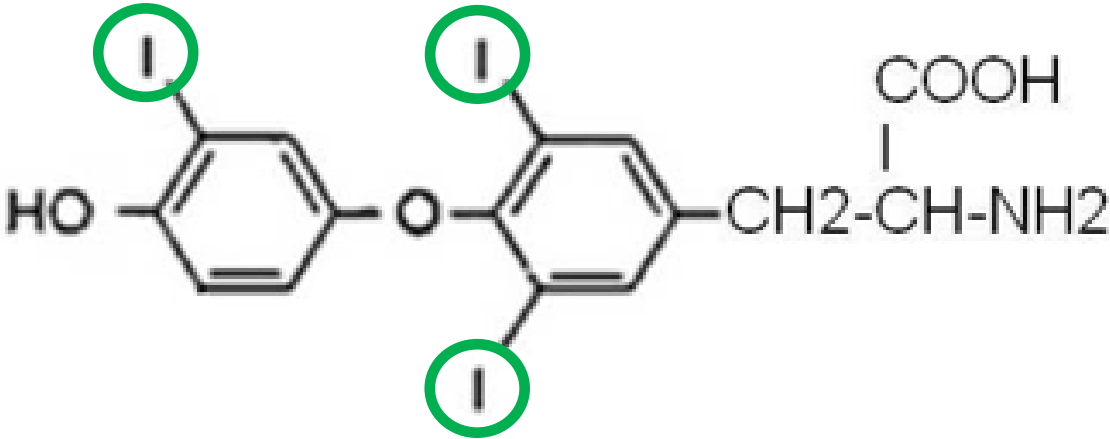
Emcitate can enter the cell without MCT8 and restore thyroid hormone signaling ✓

Discovery of *Emcitate* (Triac, tiratricol)

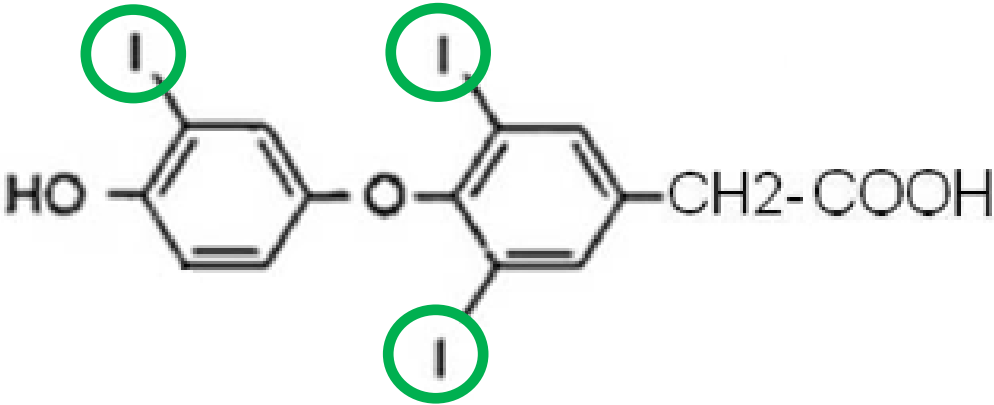


ROSALIND PITT-RIVERS
M.Sc., Ph.D. Lond.

T3



Triac
(tiratricol)



Preliminary Communication

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

Overview of completed Phase IIb – Triac Trial I

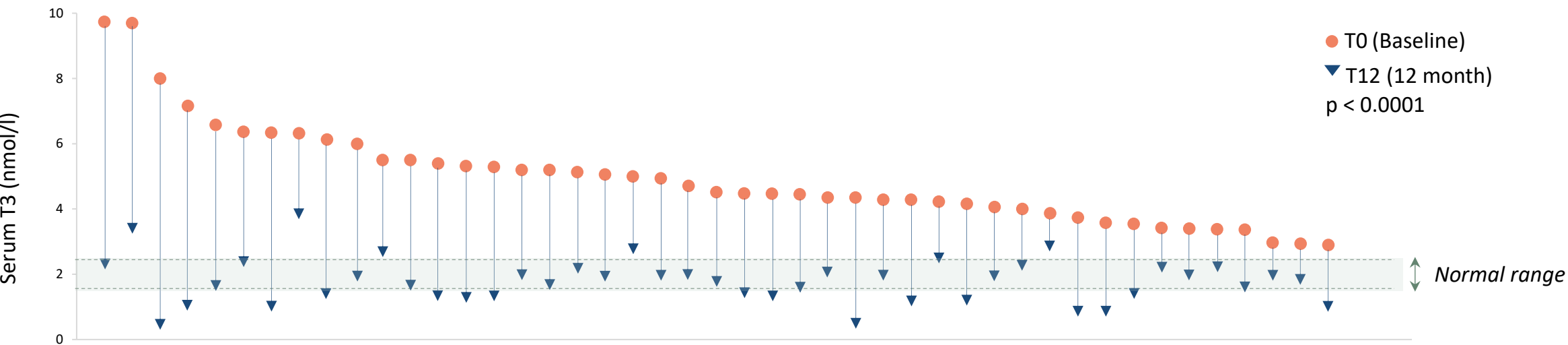
Primary objective and results	<ul style="list-style-type: none">Evaluate the efficacy and safety of oral administration of tiratricol in male patients with MCT8 deficiency of all agesHighly significant primary outcome - Change in T3 serum concentrationsSafe and tolerableResults published in <i>The Lancet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



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

Consistent, clinically relevant and highly significant results

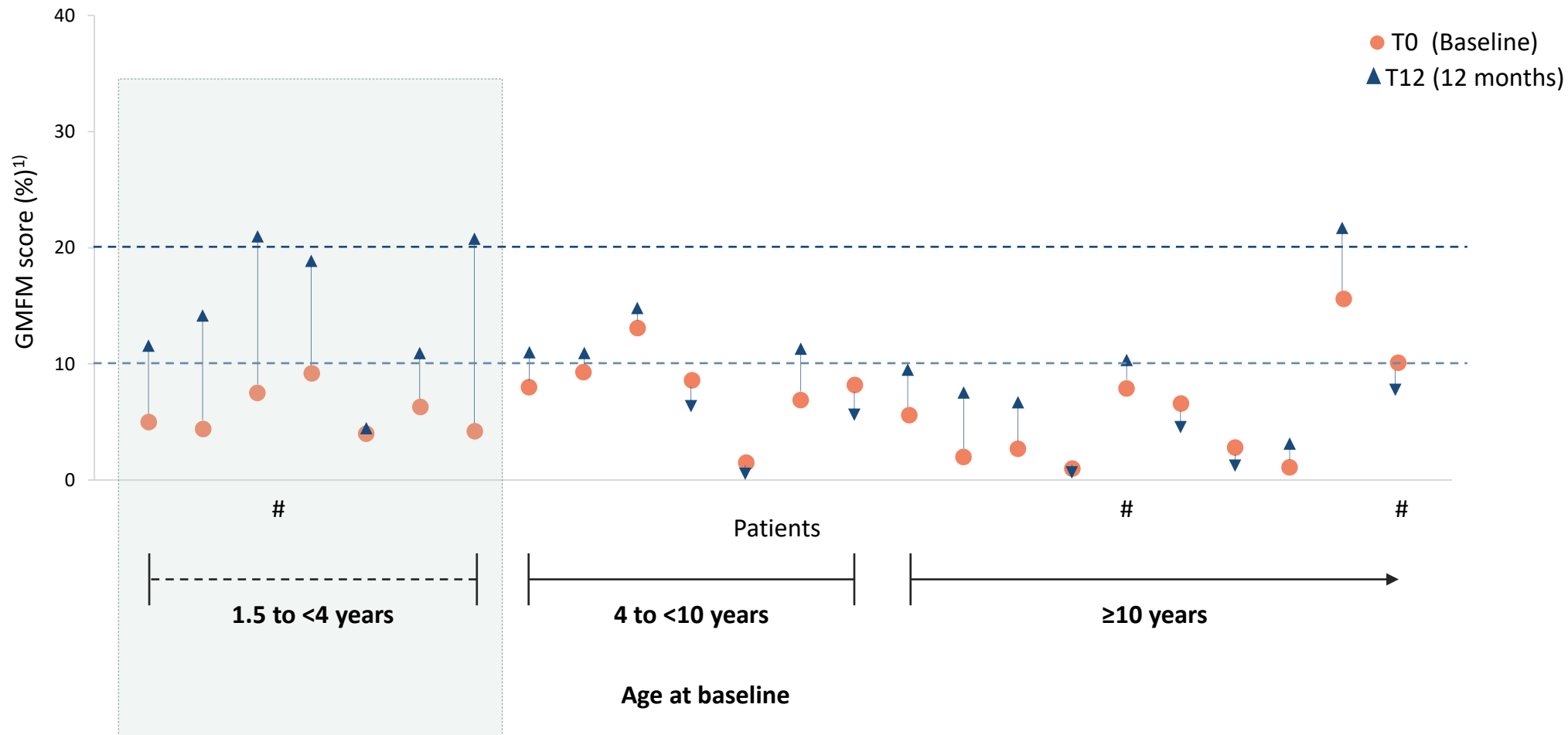
Triac Trial I: Reached target level serum T3 & improvements in clinically relevant outcome measures



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

Triac Trial I: Indication of positive effect on neurocognitive development

Triac Trial II did not meet its primary endpoints



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

Published in October, 2021

ACCEPTED MANUSCRIPT

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

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

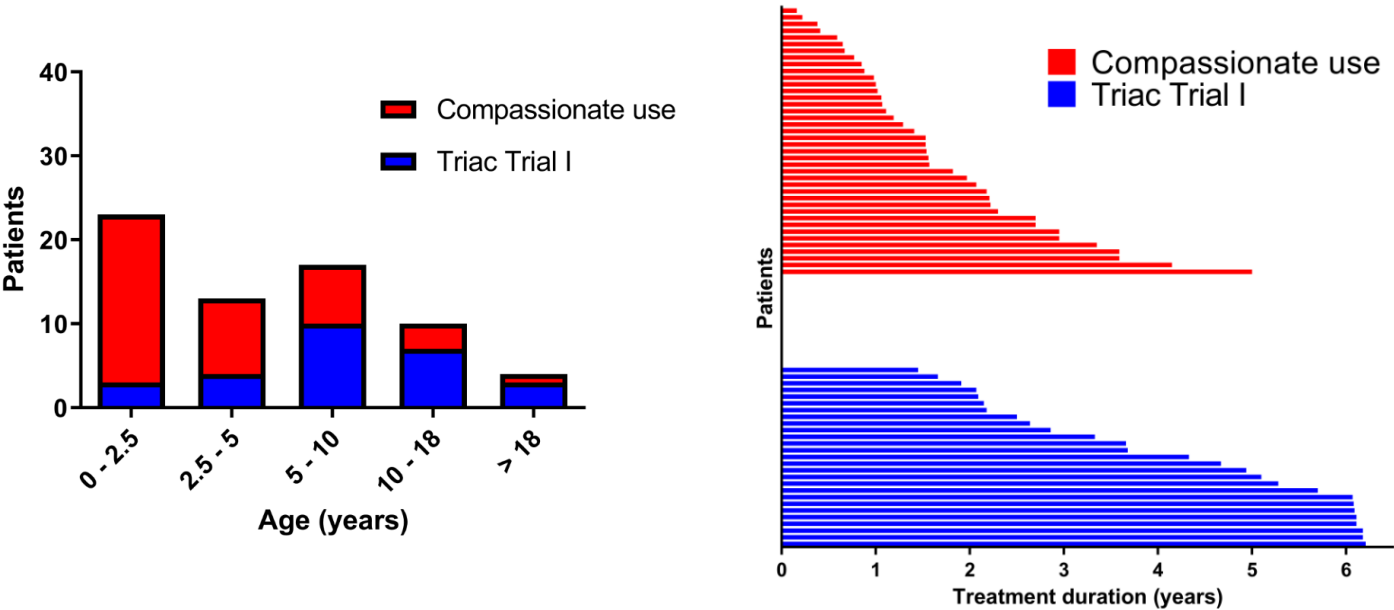
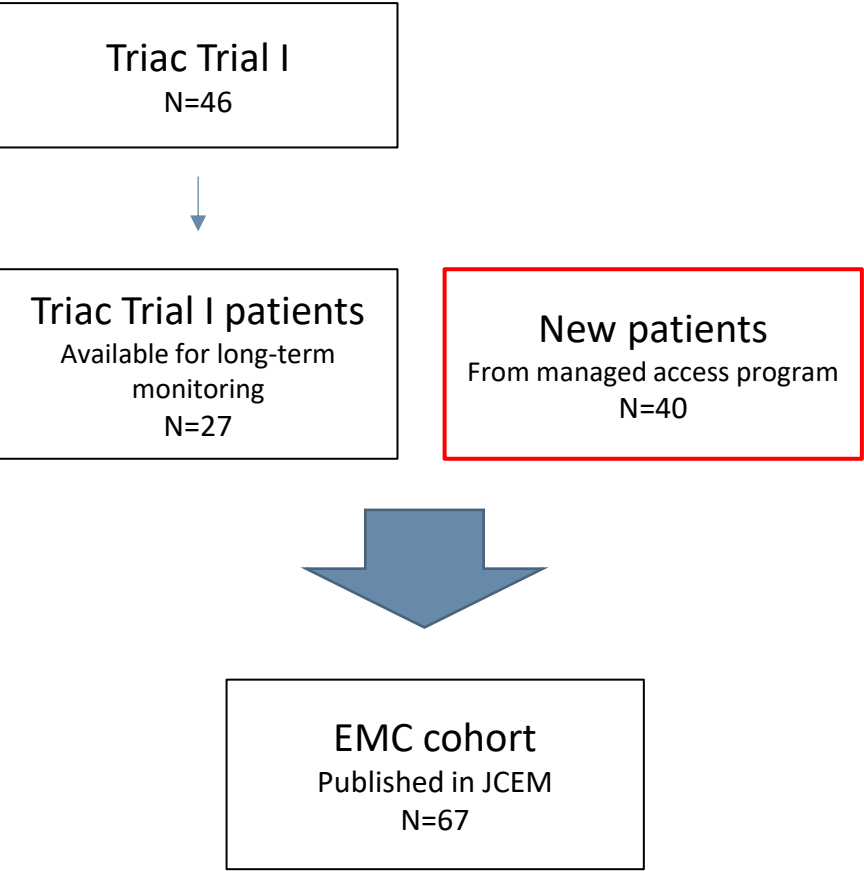
[Author Notes](#)

JCEM THE JOURNAL
OF CLINICAL
ENDOCRINOLOGY
& METABOLISM

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

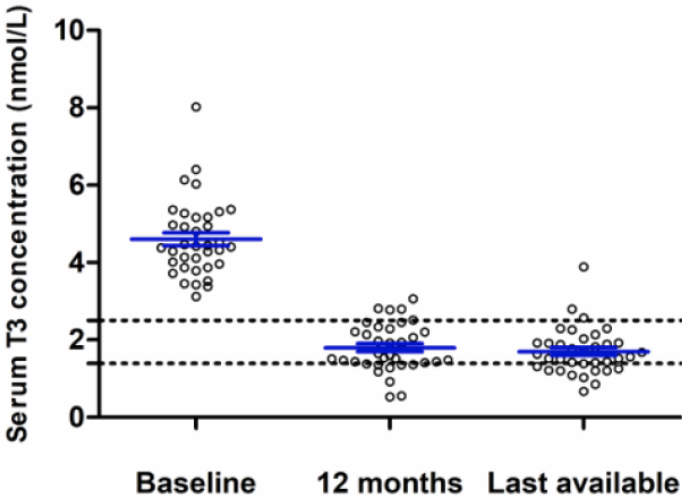
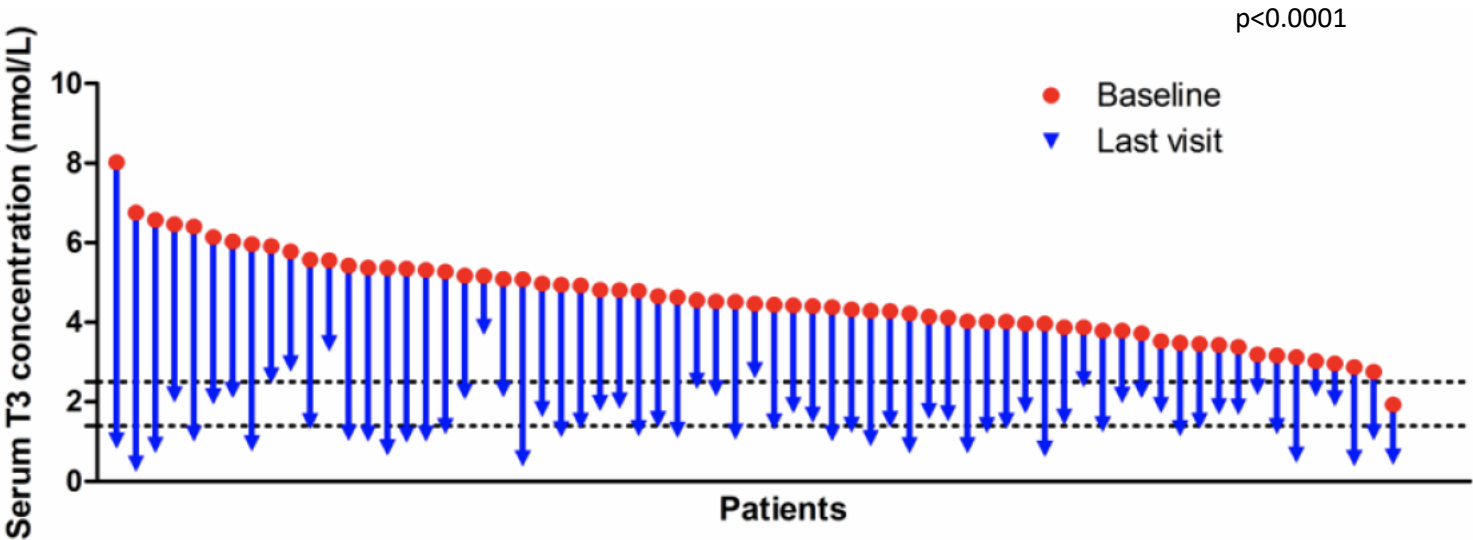
New patient cohort of equal size to the Triac Trial I

Long term follow up, up to >6 years



New cohort confirms primary endpoint results in Triac Trial I

Fast and durable normalization of T3 values in almost all patients



Consistent, clinically relevant and highly significant results across endpoints

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

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

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

Triac Trial II objective and design:

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

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



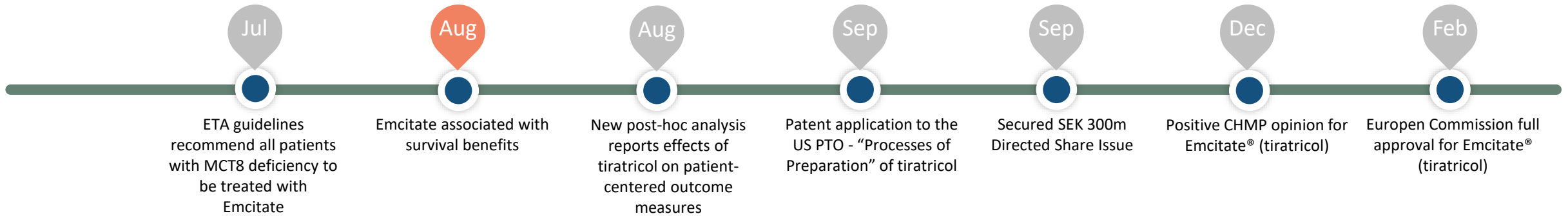
1. GMFM: Gross Motor Function Measure
2. BSID: Bayley Scales of Infant and Toddler Development

Triac Trial II Summary



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

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



- Abstract published ahead of the ETA Annual Meeting reports that treatment with tiratricol (Emcitate®) in patients with MCT8 deficiency is **associated with a 3x lower risk of mortality**.
- Retrospective real-world cohort study investigated the effects of tiratricol on mortality in 228 patients with MCT8 deficiency.



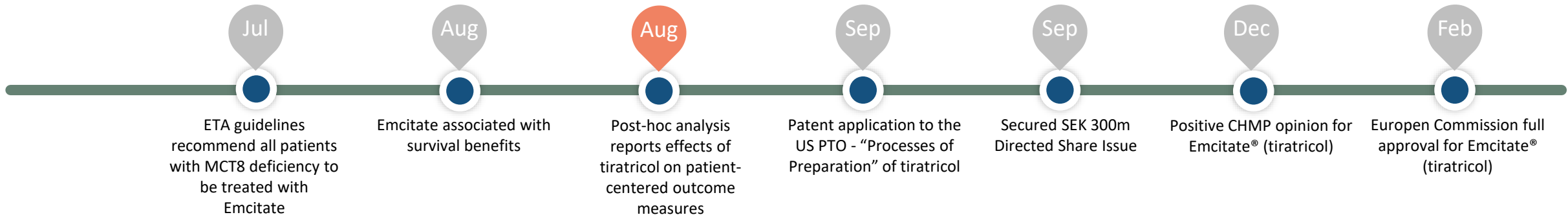
New data shows tiratricol (Emcitate®) treatment in patients with MCT8 deficiency is associated with survival benefits

August 21, 2024

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

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

Post-hoc analysis reports effects of tiratricol on patient-centered outcome measures in patients with MCT8 deficiency



- According to the Abstract, there were improvements upon tiratricol treatment reported by caregivers related to improved interaction (22/39), greater alertness (19/39), improved motor skills (12/39), improved head control (7/39), and improved sleep (8/39).
- Compared to the baseline visit, excessive sweating was much less reported (48.6% vs. 8.1%) and less reduction in salivary flow was observed (30.6% vs. 22.2%) by the caregivers at the end study visit.
- **All parents (40/40)** preferred to continue tiratricol treatment.

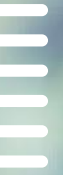


New post-hoc analysis reports effects of tiratricol on patient-centered outcome measures in patients with MCT8 deficiency

August 28, 2024

- An Abstract by Dr M. Freund and co-authors from Erasmus Medical Center, Rotterdam, The Netherlands, published ahead of the Annual Meeting of the European Thyroid Association reports that treatment with the investigational drug tiratricol exerts beneficial effects on several patient-centered outcome measures in MCT8 deficiency.

Stockholm, Sweden, August 28, 2024. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (Nasdaq Stockholm: EGTX), today announced the content of an Abstract by Matthijs Freund and co-authors, Erasmus Medical Center, Rotterdam, The Netherlands, published ahead of the 46th Annual Meeting of the European Thyroid Association, to be held in Athens, Greece, on September 7-10, 2024. In this analysis the authors performed post-hoc analyses on caregiver-reported patient-centered outcome measures in the Triac Trial I (1). In this trial, 40 patients with MCT8 deficiency completed 1 year of tiratricol treatment. At baseline, during clinical visits and at the end of the study, semi-structured interviews were held with caregivers on complex needs and daily care challenges, including motor skills, sleep problems, and seizure frequency. Moreover, parents were asked to report perceived changes in (thyrotoxic) symptoms such as increased sweating and reduction in salivary flow.



2.c

Emcitate[®] - regulatory pathways in EU and US

Regulatory features of *Emcitate* for MCT8 deficiency



ODD

Orphan drug designation for MCT8 deficiency

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

Fast
track

Fast track designation (FDA)

PRV

Rare pediatric disease designation (FDA)

Eligibility: Priority review voucher upon approval*

MAA
NDA

MAA: EU full approval received in February 2025

NDA: Pivotal ReTRIACt study in at least 16 evaluable patients ongoing



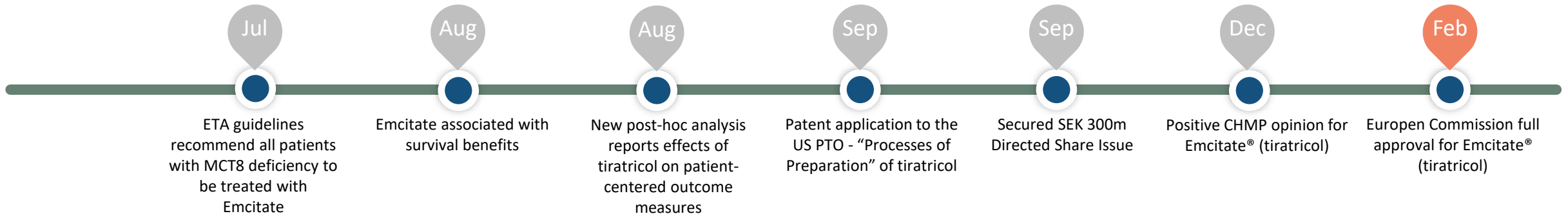
ODD

Orphan drug designation for RTH-beta

*The voucher may be sold to another sponsor (2023-25 range: ~\$100m-\$158m)

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EU Commission approves Emcitate® as the first and only treatment for patients with MCT8 deficiency



- **Full marketing authorisation.**

“This is the single most important milestone in Egetis’ history and a major step forward in building a sustainable rare disease company”



European Commission approves Egetis’ Emcitate® (tiratricol) as the first and only treatment for patients with MCT8 deficiency

February 13, 2025

Stockholm, Sweden, February 13, 2025. Egetis Therapeutics AB (publ) (“Egetis” or the “Company”) (Nasdaq Stockholm: EGTIX), today announced that the European Commission (EC) has approved Emcitate® (tiratricol) for the treatment of patients with monocarboxylate transporter 8 (MCT8) deficiency. Emcitate is the first and only medicine authorised in the EU to treat MCT8 deficiency. The full indication is: Emcitate is indicated for the treatment of peripheral thyrotoxicosis in patients with monocarboxylate transporter 8 (MCT8) deficiency (Allan-Herndon-Dudley Syndrome), from birth.

Nicklas Westerholm, CEO of Egetis, commented: “We are proud of the European Commission approval of Emcitate, which marks the first and only approved treatment for patients with MCT8 deficiency. This approval represents the single most important milestone in Egetis’ history and a major step forward in building a sustainable rare disease company. We are delighted to bring this much needed new treatment to patients.

“I would like to thank all patients, parents, caregivers and investigators who have taken part in the comprehensive development program for Emcitate and all Egetis employees and collaborators for their dedicated and hard work, in particular the group of Prof. Dr. Edward Visser at the Erasmus University Medical Center, Rotterdam, The Netherlands.

“We look forward to initiating pricing and reimbursement processes and discussions in Europe and expect the first launch in the second quarter of 2025.”

Emcitate/tiratricol regulatory pathway in EU/US

Robust data set in an ultra rare genetic disease



Triac Trial I	EMC cohort study	Natural history	Triac Trial II	Survival study		ReTRIACt Trial
				Abstract at ETA (EMA)	Full data set (FDA)	
N=46	N=67	N=151	N=22	N=228	N>228	N=16
<ul style="list-style-type: none">Completed 2018 (Groeneweg, 2019)Open-label, international, multi-centre study	<ul style="list-style-type: none">Completed 2021 (van Geest, 2022)N= 27 from Triac Trial I & N= 40 new pts from managed access program	<ul style="list-style-type: none">Retrospective data, 2003 to 2019 (Groeneweg, 2020)	<ul style="list-style-type: none">Open-label, international, multi-centre studyFocus on neurocognition; did not meet its primary endpoints96 weeks safety data in young patients	<ul style="list-style-type: none">International real-world cohort studyComparing treated vs untreated patients on survivalTreatment with tiratricol (Emcitate®) is associated with a 3x lower risk of mortality.	<ul style="list-style-type: none">N= 16Placebo-controlled withdrawal studyOngoing	

Data included in EMA MAA – EC approval Feb 2025

Data to be included in FDA NDA

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

Pivotal randomized placebo-controlled trial for NDA submission



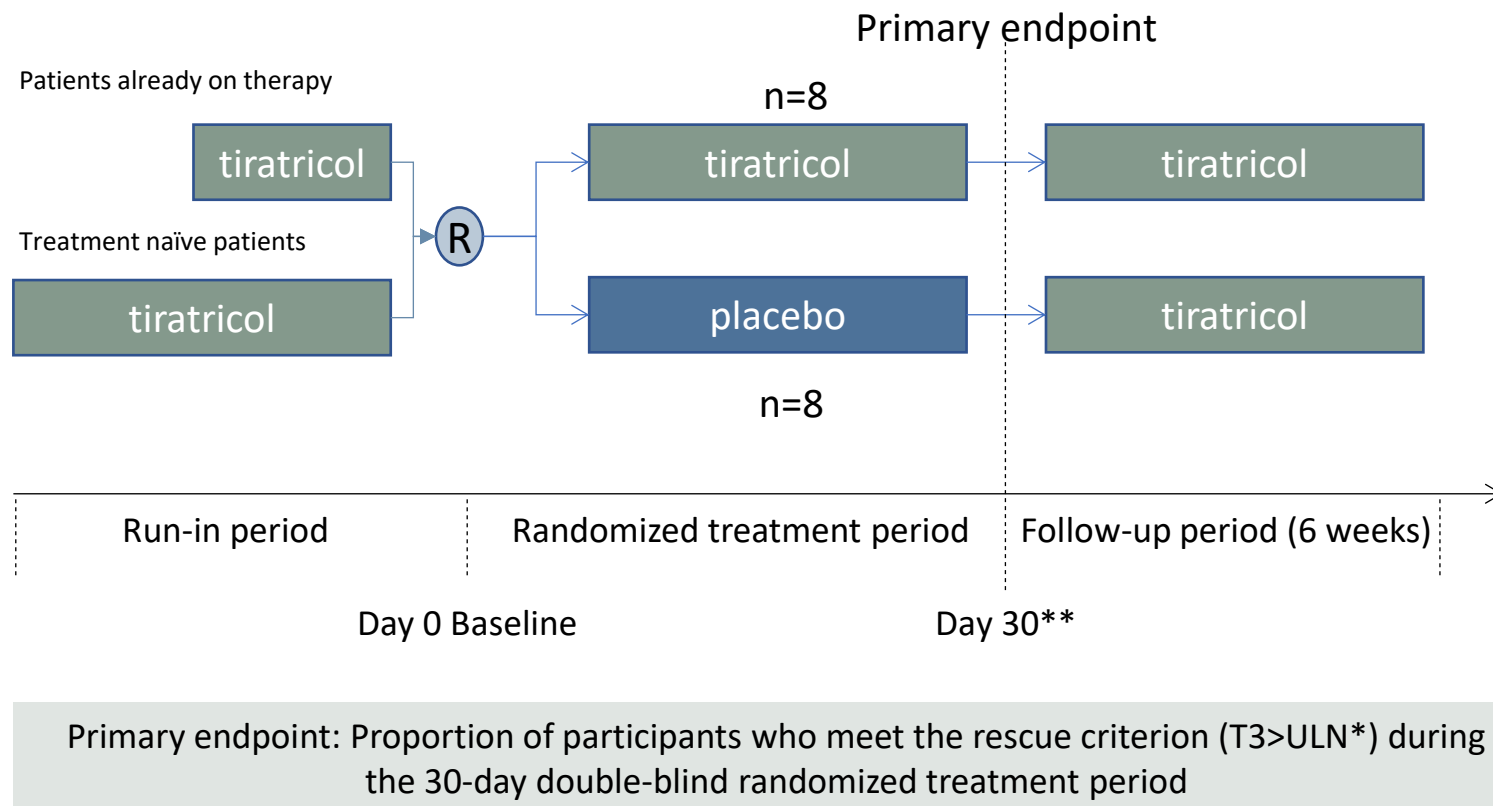
Primary endpoint	<ul style="list-style-type: none">• Proportion of participants who meet the rescue criterion (serum total T3 > ULN) during the 30-day double-blind Randomized Treatment Period
Secondary endpoints	<ul style="list-style-type: none">• Change in cardiovascular variables• Change in serum thyroid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



Design of the ReTRIACt clinical trial

Requested by the FDA

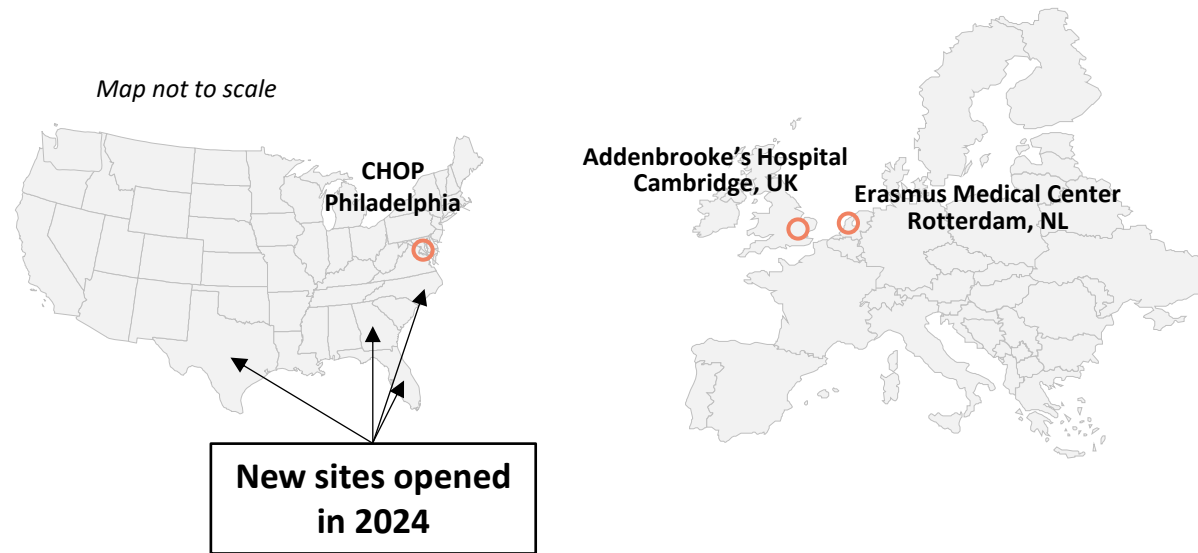
- A 30-day, randomized placebo-controlled withdrawal study in 16 patients
- Design agreed with FDA
- The study allows for inclusion of patients that are already on therapy and patients that are treatment naïve
- Treatment naïve patients require a longer run-in period to stabilize T3 levels around normal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



* ULN: Upper Limit of Normal

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

Current status of ReTRIACt trial (as of Apr 30, 2025)



- 12 patients have completed the randomized phase, 1 patient is in the randomized phase and 1 patient is in the run-in period. Two patients are planned for screening and additional patients are being evaluated for screening.
- 7 sites currently open, including the sites in Georgia, North Carolina, Texas and Florida.
- Recruitment will continue until at least 16 patients have completed the randomized phase.

⇒ Egetis will update the market as soon as recruitment has been completed, and subsequently when top-line results are expected. NDA filing is planned for 2025.

Upcoming value enhancing key milestones 2025-2026



Emcite®

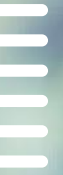
2025-2026

MCT8
deficiency

- EU launch, in the first country, Germany, May 1, 2025
- Topline results ReTRIACt for US NDA
- Submission of US NDA – priority review
- Middle East, North Africa & Turkey partnership/s
- Japan – Development plan agreed with PMDA
- US Patent granted - Processes and compounds
- US approval and launch
- US Rare Pediatric Disease Priority Review Voucher

RTH-beta

- Potential initiation of Investigator Initiated Study - Egetis Industry collaborator



2.d

Emcitate[®] - Commercial opportunity

Emcite[®] – alleviating patient and societal burden

Aiming to provide value for both patients and society

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

Patients

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

Society

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



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

Emcitate supplied globally in managed access programs

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

- Managed access programs
 - mechanisms to allow early access to a medicine prior to regulatory marketing approval
 - granted to pharmaceuticals under development for situations with high unmet medical needs and where no available treatment alternatives exist or are suitable
- FDA approved Expanded Access Program - Simplifies Process for Accessing *Emcitate*
- *Emcitate* is being supplied in managed access programs, following individual approval from the national medicines agencies, to
 - Around 230 patients
 - Over 25 countries



Patient

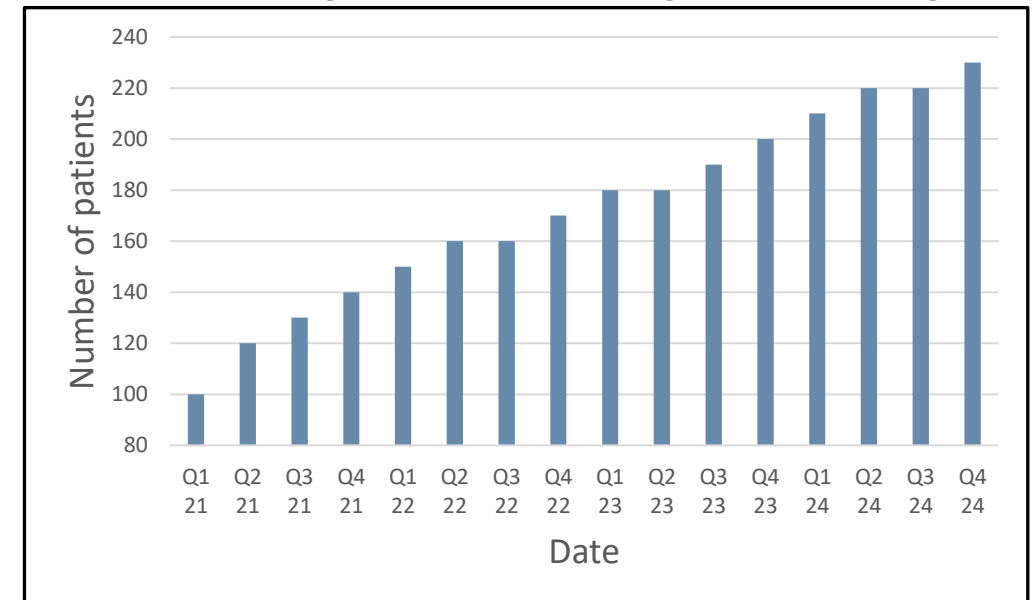


Prescriber



National Approval

Patients Receiving Emcitate in Managed Access Programs



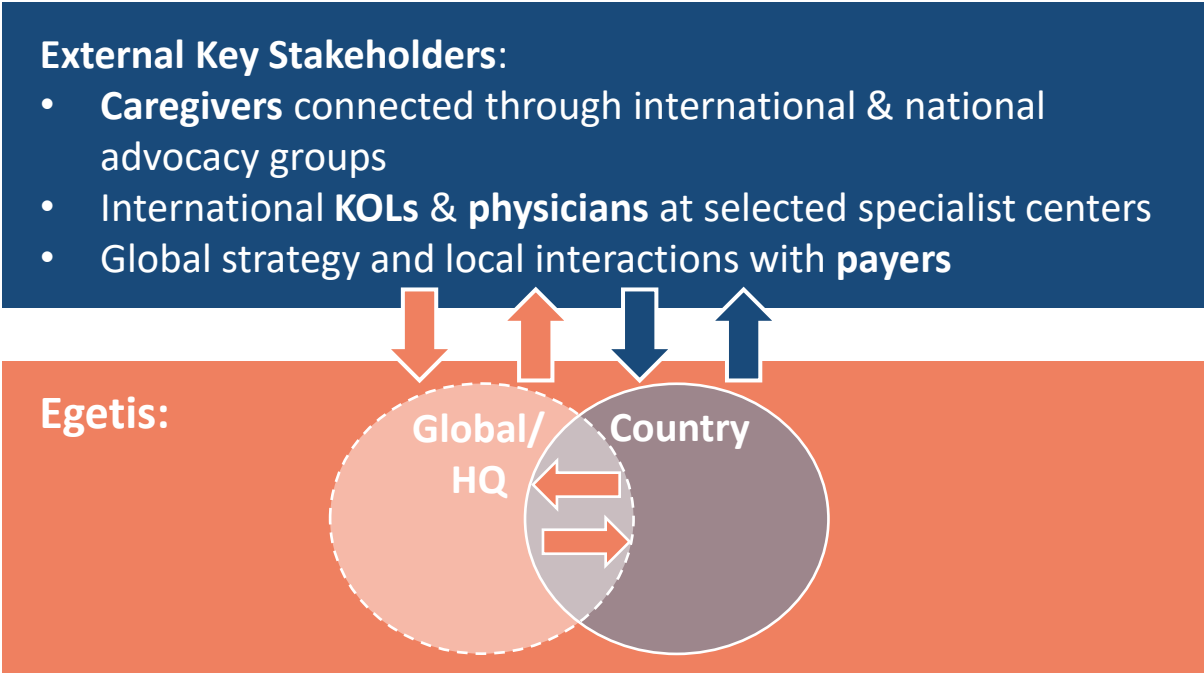
Commercialization possible with lean & agile team

Unique setting for Emetate in MCT8 deficiency



Seizing opportunity for cost-effective value creation

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



Preparing for *Emcitate* launch by Egetis and partners

Executing the US & European market preparations and launches through the Egetis team

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

Optimizing additional countries through partners

MENAT partnering dialogues

Japan license deal with Fujimoto

Step-wise building team 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

– Core team brings launch skills and best practices from in total 150+ years at international companies



Henrik Krook, SE
VP, Commercial Operations



Anny Bedard, US
President Egetis North America



Henna Oittinen Corbinelli, CH
Medical Director Europe & International



Ann-Marie Redmond, US
Head of Market Access & Pricing,
North America



Nadia Georges, CH
Global Head, Market Access & Pricing



Azza Trad, FR
GM France



Susana Roche, FR
Associate Director Global
Medical Affairs Operations



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



Peter Verwaijen, NL
Global Head Brand Strategy &
Commercial Business Expansion,
GM Benelux



Raymond Francot, NL
GM for DACH, IT,
Central & Eastern Europe



Focusing on Critical Areas for Launch Success



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

IDENTIFY PATIENTS

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



ENSURE ACCESS

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



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

Expanding disease awareness momentum

Amplified by External Efforts



Constructive dialogues at scientific congresses



Scientific community generating more data

Example from Annual Meeting of the European Thyroid Association

Van der Most, F. et al. T3 analogue Triiodothyroacetic acid (Triac) treatment and survival in MCT8 deficiency: an international real-world cohort study

Freund, M. et al. Effect of the T3 analogue Triac on patient-centered outcome measures in patients with MCT8 deficiency: post-hoc analysis of the international Triac Trial I

5 additional abstracts related to MCT8 deficiency

Great work ongoing by several patient advocacy groups



Deliver solid *Emcitate* clinical and economic value proposition to enable reimbursement & broad access

Key for payer assessments to describe burden of disease, unmet need & benefit of treatment

High burden of MCT8 deficiency

Recently further supported by Egetis sponsored Caregiver study*

Caregiver-reported quality of life of patients with MCT8 deficiency: Results from a cross-sectional survey

Introduction

Microsomal transporter 8 (MCT8) deficiency is a rare autosomal recessive disorder with the clinical picture of severe intellectual disability and growth retardation. It is caused by mutations in the MCT8 gene, which encodes a protein that transports thyroid hormones across the blood-brain barrier. The resulting deficiency of thyroid hormones in the brain leads to the characteristic clinical features.

Methods

An international survey of caregivers of patients with MCT8 deficiency was conducted. The survey aimed to assess the burden of the disease on caregivers and the impact of the disease on the patients' quality of life. The survey included questions about the caregivers' demographic information, the patients' clinical features, and the caregivers' perceived burden of the disease.

Results

The survey included 10 caregivers from 10 different countries. The results showed that the caregivers reported a high burden of the disease, with significant impacts on their daily lives and the patients' quality of life. The patients' clinical features were consistent with the known clinical picture of MCT8 deficiency.

Conclusion

The results of the survey highlight the need for further research and clinical trials to improve the management of MCT8 deficiency. The survey also provides valuable information for the development of patient support programs and clinical trial recruitment.

References


1. [Reference 1]

2. [Reference 2]

3. [Reference 3]

Significant unmet medical need

Currently no drug developed and regulatory approved for MCT8 deficiency



Emcitate benefit validated by physicians and regulators

The existing clinical experience and data contributed to:

- European Thyroid Association (ETA) recommending *Emcitate* as long-term therapy for all patients with MCT8 deficiency
- Positive CHMP opinion

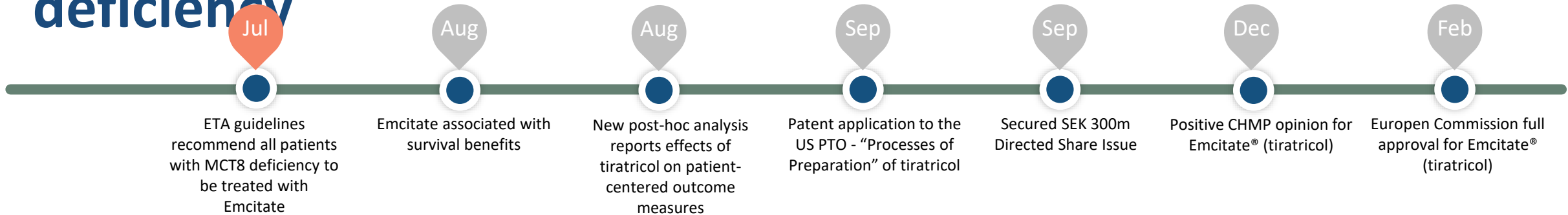
* Posters presented at congresses 2024, at ESPE (European Society of Pediatric Endocrinology) and ISPOR (International Society for Pharmacoeconomics and Outcomes Research).

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



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



A phased EU launch through in-house commercial organization starting in Germany May 1

Pricing & Reimbursement (P&R) strategy execution in 2 waves, starting with EU4

Wave 1

France, Germany, Italy, Spain



Wave 2

Phased on a country-by-country approach

Rest of Europe



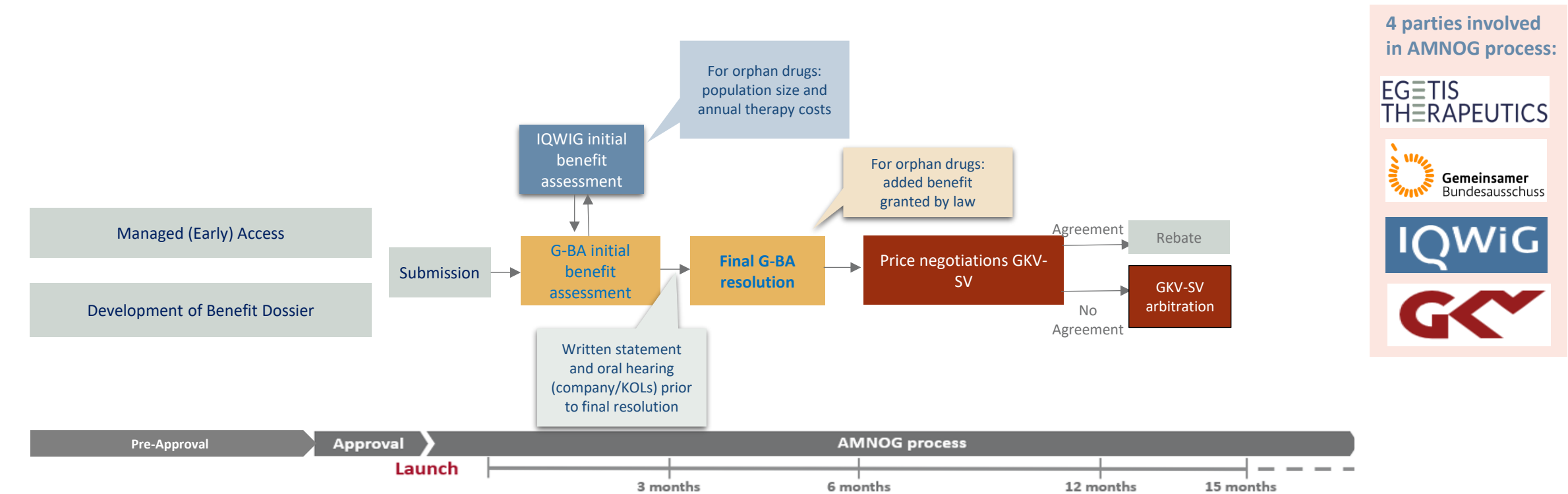
Pricing &
Reimbursement
processes

Deliver the *Emcitate* clinical and economic value proposition in P&R processes, outlining:

- MCT8 deficiency and its rarity
 - Summarizing available literature
- High burden of MCT8 deficiency
 - Confirmed by Egetis sponsored Caregiver study
- Significant unmet medical need
 - Emcitate the first & only approved treatment
- Benefit of treatment
 - Supported by publications & ETA guidelines

Germany: Benefit assessment and price negotiations for new drugs follow a strict and transparent process

AMNOG Process is well-defined and led by G-BA for benefit assessment and by GKV for price negotiations



G-BA: Gemeinsamer Bundesausschuß - Federal Joint Commission
GKV-SV: Gesetzliche Krankenversicherung Spitzenverband - Statutory Health Insurance
IQWiG: Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen – Institute for quality and Efficiency in Health Care
KOLs: Key Opinion Leaders

Germany Launch Strategy

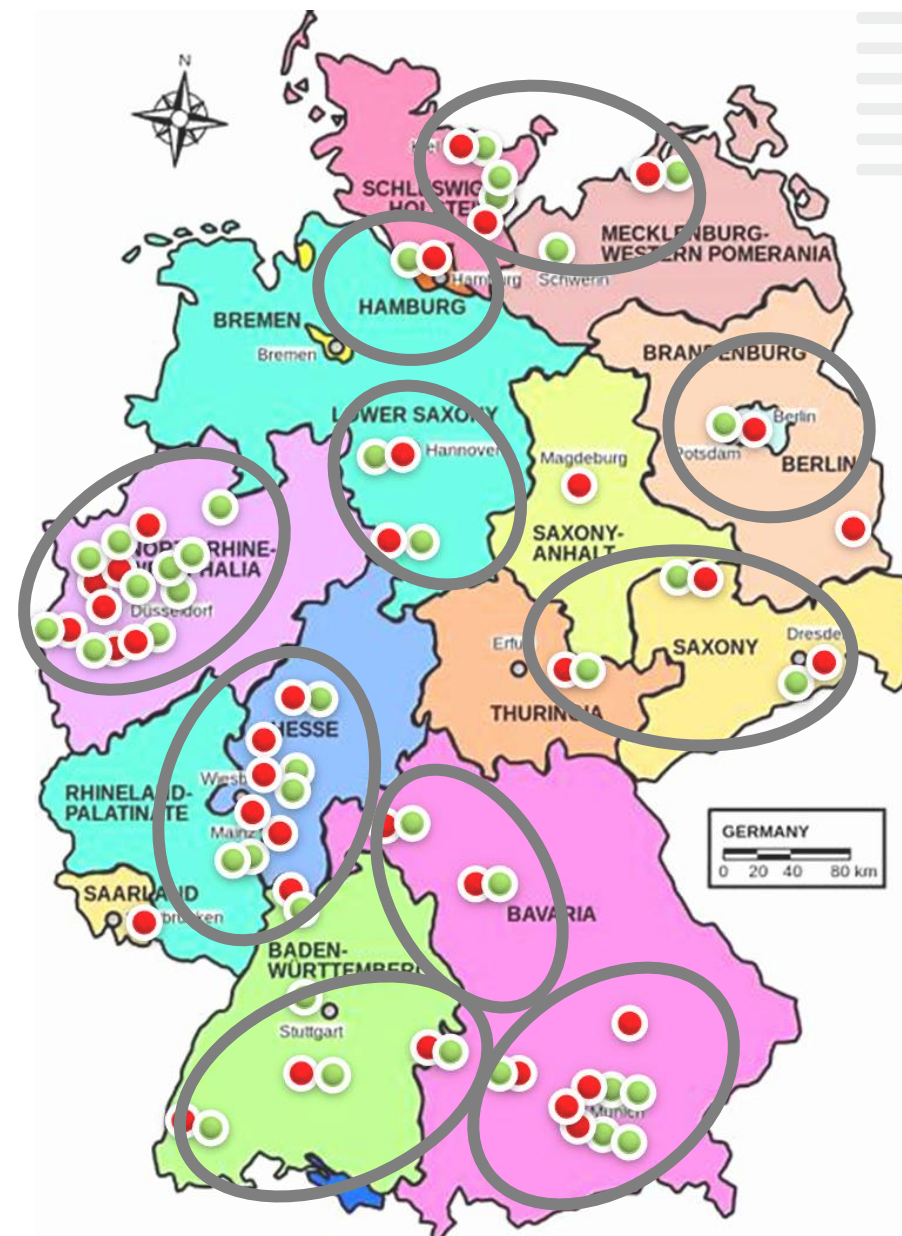
Building strong Expert base to advance management of MCT8 deficiency

MCT8 deficiency Experts

- Engage experts in increasing disease awareness in Germany
- Advance collaborative efforts on monitoring and treatment guidance of MCT8 deficiency
- Advocate for importance of local publications & clinical training in managing MCT8 deficiency

HCPs involved in patient journey

- Collaborate with SPZs and ZSEs involved in MCT8 deficiency patient journey and subsequent disease management
- Increase disease awareness and encourage discussions in local educational training sessions in multidisciplinary HCP teams
- Develop customized awareness campaigns to HCPs as well as patient support materials in collaboration with disease experts



Entering new phase with EU approval and Germany launch

As of May 1: Easier access to Emcitate and promoting Emcitate e.g. at congresses

Congresses, H1	Geogr. scope	When
APS	Germany	March ✓
Deutsche Gesellschaft für Endokrinologie (DGE)	Germany	March ✓
DACH Kongress für seltene Erkrankungen	DACH	April ✓
APEDÖ	DACH	April ✓
Joint Congress of ESPE and ESE 2025	Europe	May
Luisenthaler Gespräche (Endocrinology)	Germany	May

NEU BEI
MCT8 DEFIZIENZ

HELFE SIE IHREN PATIENTEN,
DIE RISIKEN
DER THYREOTOXIKOSE
ZU ÜBERWINDEN

NEUESTE ETA-LEITLINIEN
EMPFEHLEN EMCITATE®
FÜR ALLE PATIENTEN MIT
MCT8 DEFICIENCY

EMCITATE®, die erste und einzige zugelassene Behandlung für
MCT8-defizienz, verringert nachweislich Risiken der Thyreotoxikose¹⁻³

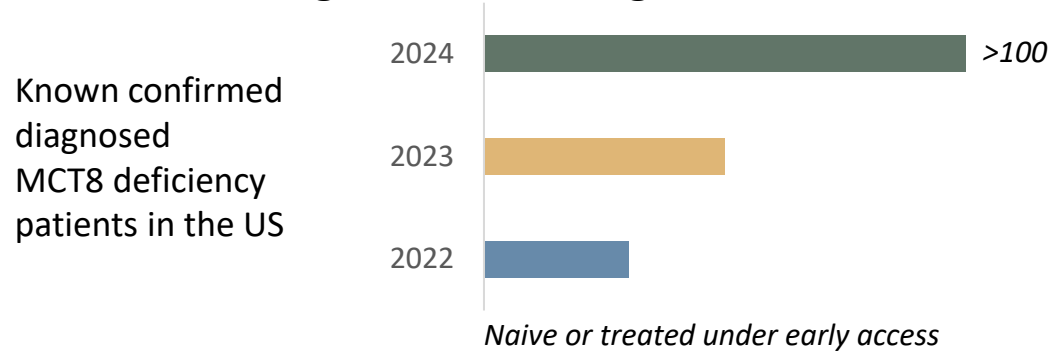
- Wirkt gezielt gegen die Grundursache der Thyreotoxikose bei MCT8-defizienz, einer seltenen, schwer beeinträchtigenden X-chromosomalen Erkrankung¹⁻⁴
- Reduziert lebensbedrohliche Symptome wie Herzrhythmusstörungen und Untergewicht in einer Erkrankung, bei der 1 von 3 Kindern das Erwachsenenalter nicht erreichen¹⁻⁴
- Mild, die tägliche Belastung für Patienten und deren Betreuer zu verringern²
- Geeignet für alle Patienten mit MCT8-defizienz²

EMCITATE®
Tiratricol
Risiken der Thyreotoxikose reduzieren¹

Draft for MLR review

US commercial preparedness activities under way

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



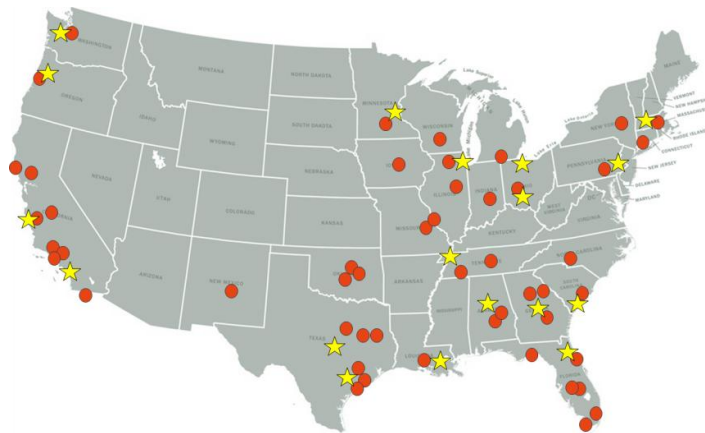
Expanded Access Program, requested by FDA, is a significant asset to both patients and Egetis launch readiness

- Providing early and sustainable access to therapy
- Physicians gain experience of tiratricol
- Opportunity to generate real world data to support regulatory and payer interactions

Cover major US centers through pre-commercial initiatives

● Hospitals with known MCT8 deficiency patients

★ Tiratricol Expanded Access Program sites, active & in process



Patient-centric implementation

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

Disclaimer: tiratricol is investigational and has not been approved by the U.S. Food and Drug Administration (FDA). Its safety and efficacy have not been established for the US market.

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



Tiratricol (Emcitate) Expanded Access Program sites



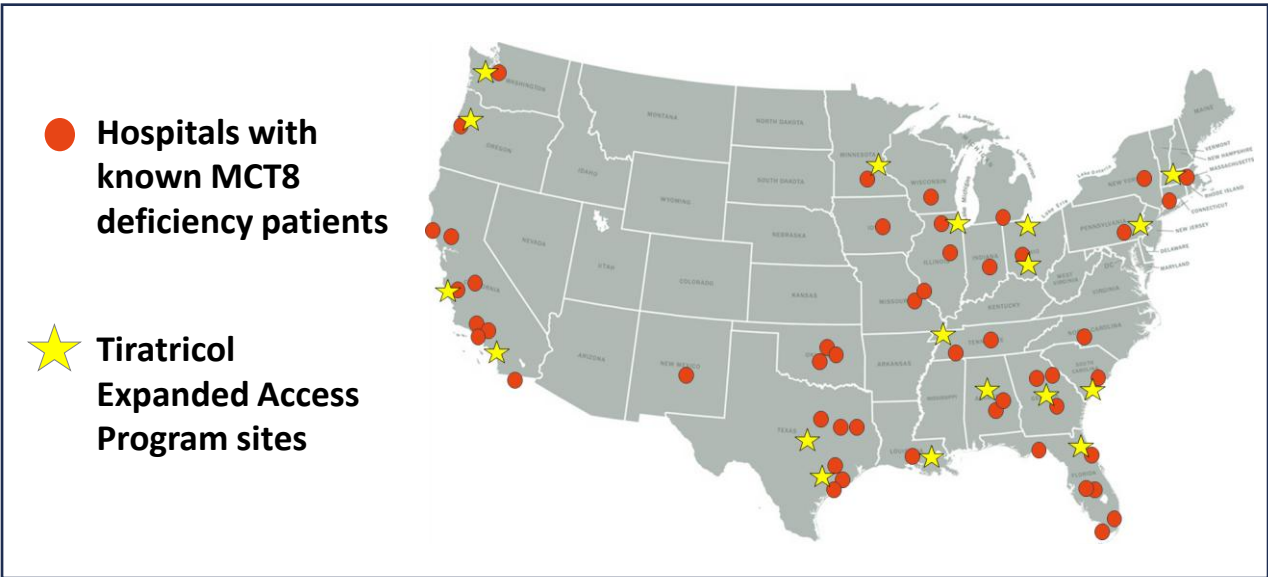
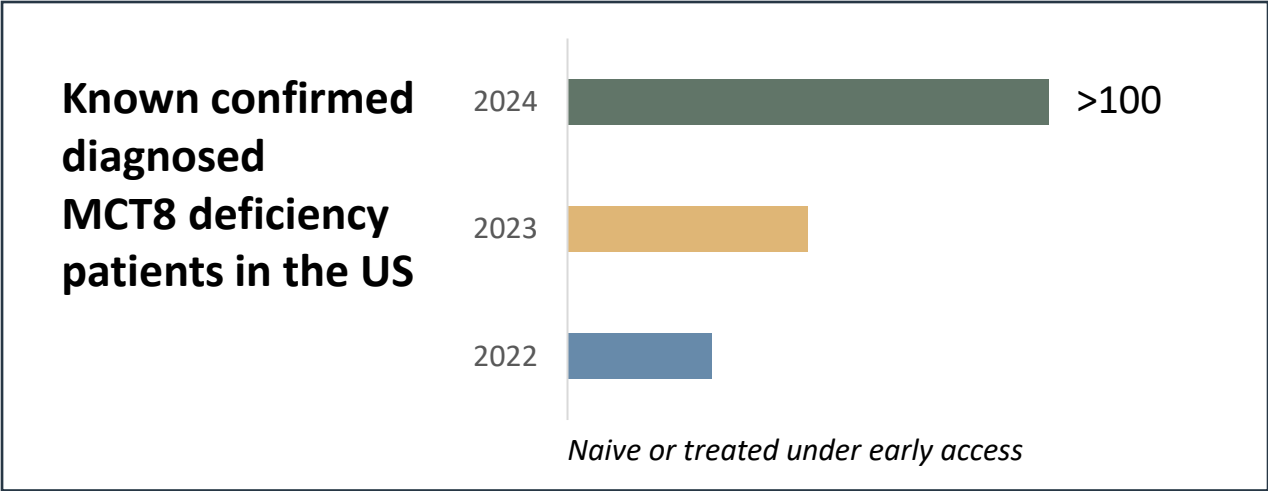
A significant asset to both the patients and Egetis launch readiness

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

Patient-centric implementation

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

Disease awareness activities in the US are bearing fruit



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

Balancing Annual Treatment Costs and Broad Access



Analogues

<u>Product</u>	<u>Disease</u>	<u>Estimated annual treatment cost (WAC)</u>
Skyclarys® <i>Small molecule</i>	Friedreich ataxia	~\$400K
Procysbi® <i>Small molecule</i>	Nephropathic cystinosis	~\$550K
Ravicti® <i>Small molecule</i>	Urea cycle disorder	~\$750K
Exondys® <i>Antisense oligonucleotide</i>	Duchenne Muscular Dystrophy	~\$750K



Access

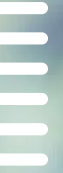
Less restrictive

- Prior Authorization to label
- Genetic Test Attestation/documentation
- Specialist prescribing



More restrictive

- Prior Authorization beyond label
- Attestation of clinical benefit
- Medical exception with appeal



2.e

Emcitate partnerships

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



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



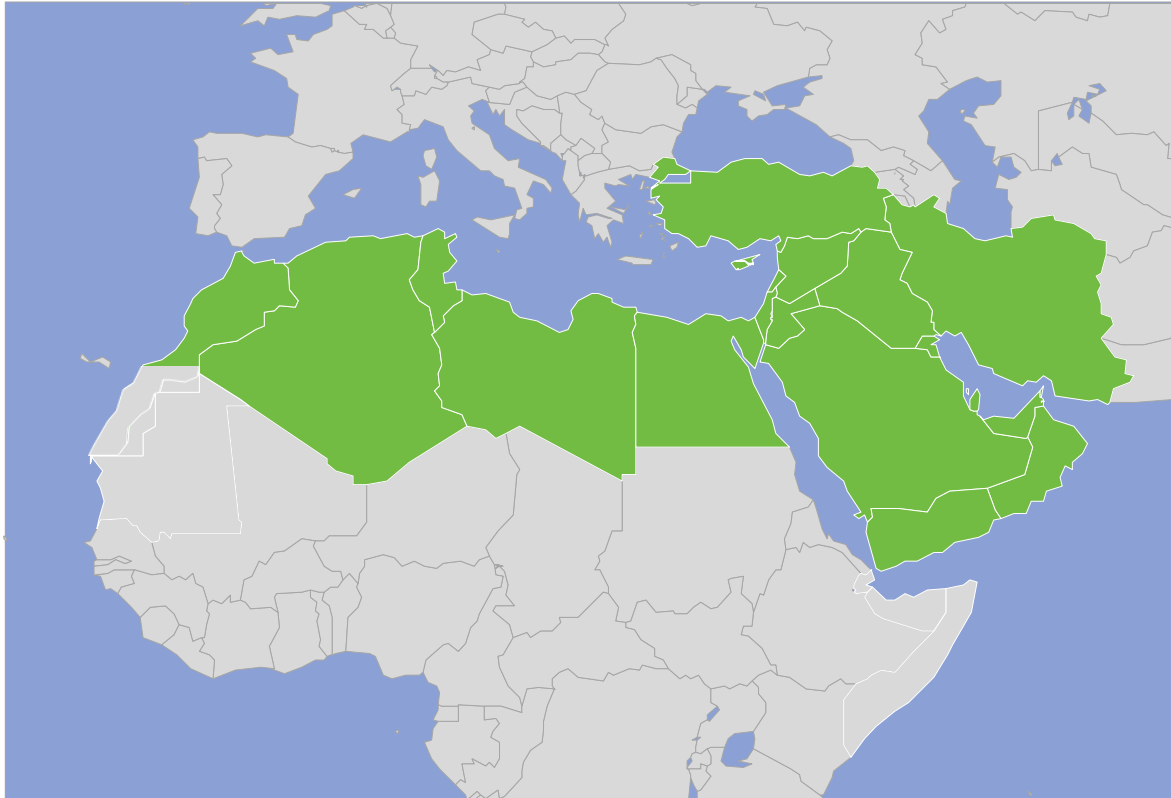
Egetis announces exclusive license agreement with Fujimoto to develop and commercialize Emcitate in Japan

November 10, 2023

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

The MENAT-region

Opportunity for patient access based on EMA approval in the Middle-East, North-Africa and Turkey



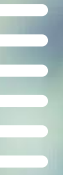
- MENAT-region has a large population with well established healthcare systems
- EMA approval allows for access in some of the countries without the need for national regulatory submissions
- Different healthcare systems require local knowledge and expertise

Egetis' approach to the MENAT-region

Serving patients in the MENAT-region by working together with local partners



- Given that Europe and the US are the priorities for Egetis together with the need for local resources in the MENAT-region, Egetis is currently identifying strategic partners for collaboration and access
- Important criteria for the selection are:
 - Proven track record and reputation
 - Experienced in providing access for rare diseases
 - Full set of functions (Regulatory, Market Access, Medical Affairs, Commercial, Supply Chain and Pharmacovigilance) with local representatives
 - Committed to deliver the value of Emcitate® to patients in the region
- Egetis' ambition is to sign the first partnership agreement for MENAT in 2025



3.

Potential for indication expansion into RTH-beta

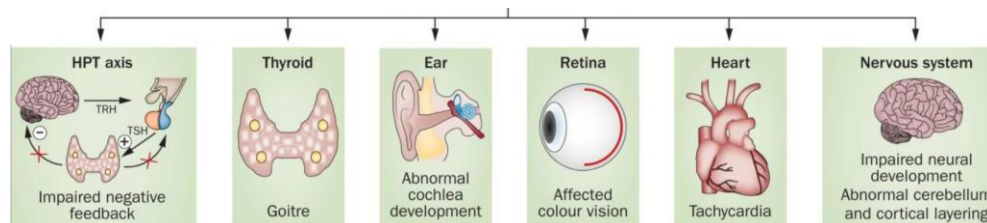
Resistance to Thyroid Hormone type Beta (RTH-β)

Potential indication expansion for Emcitate into non-overlapping patient population

Characteristics of RTH-β

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

Overview of tissues affected in RTH-β



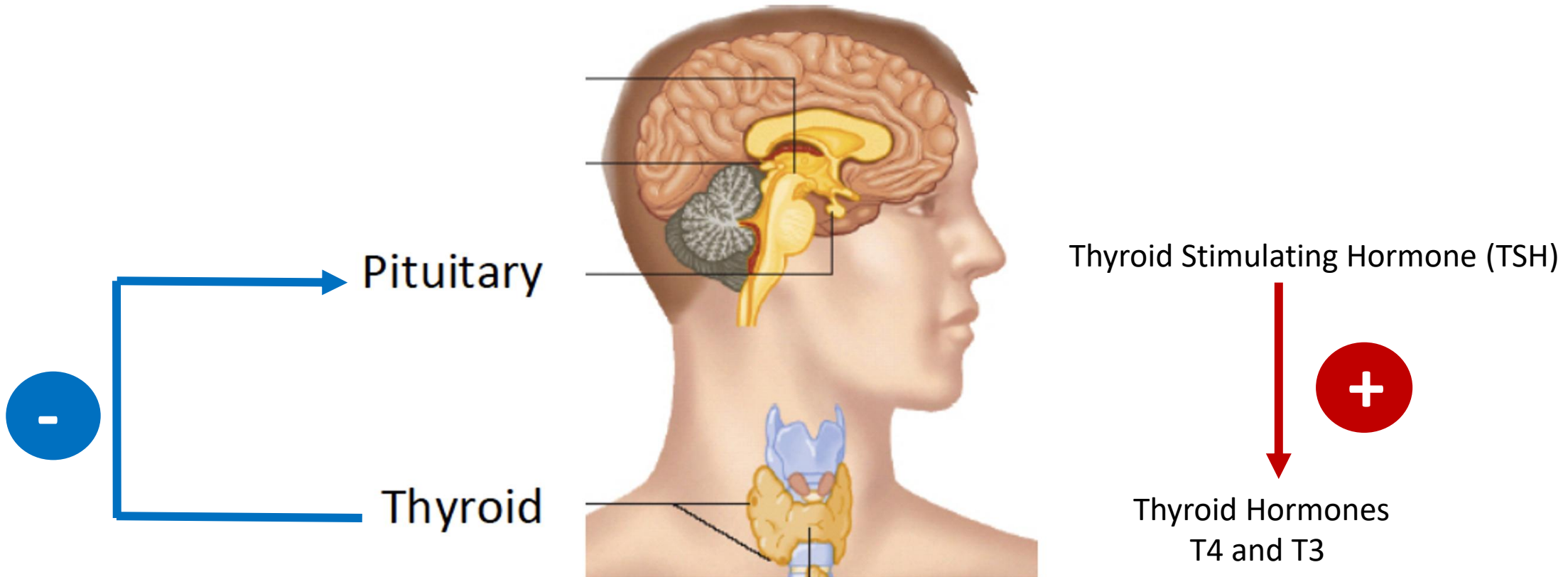
Emcitate as potential treatment for RTH-β

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

References:

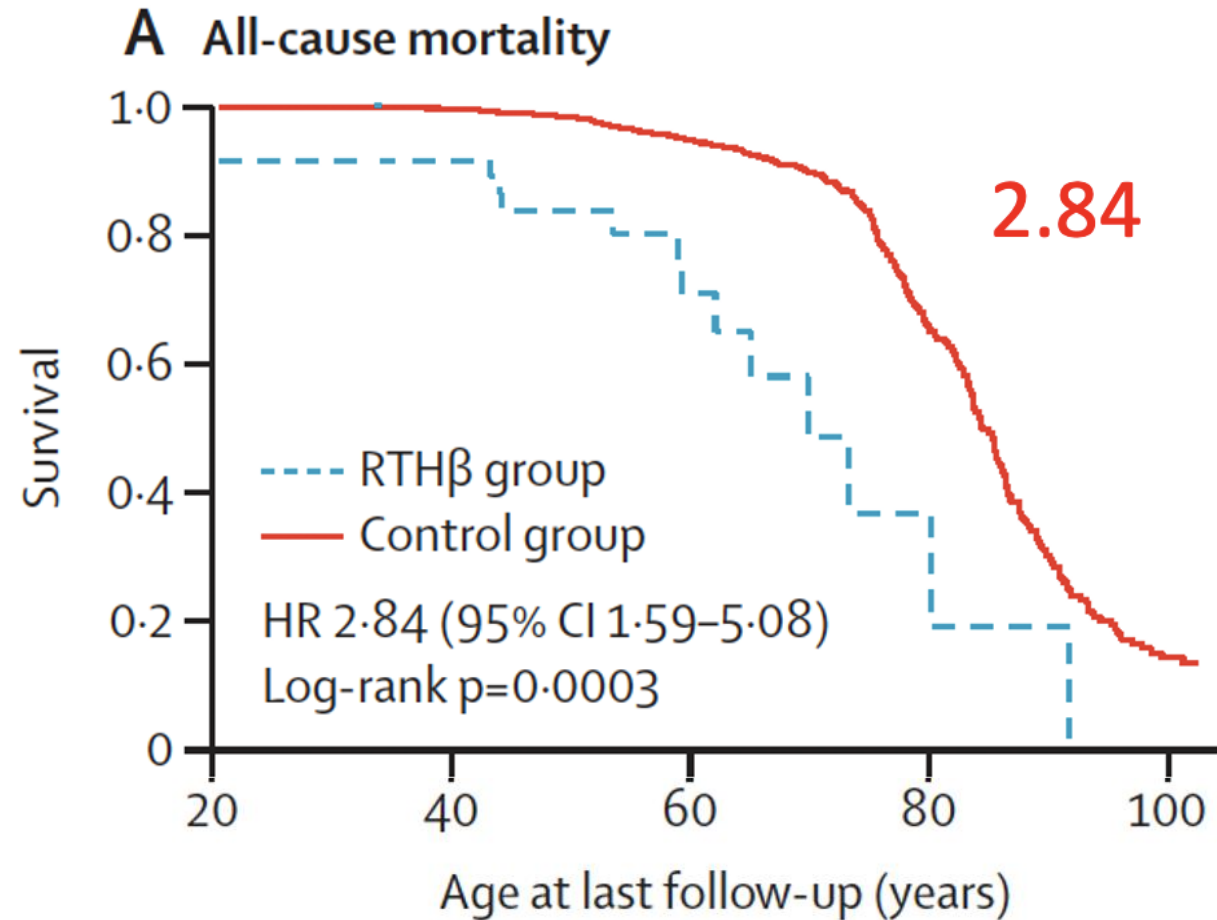
1. Pappa & Refetoff (2021) Front. Endocrinol. 12, 656551
2. Anzai et al. (2012) Thyroid 22, 1069-1075

“The Feedback Loop” in RTH β



		Example levels	Normal Levels
TSH	NORMAL RANGE	4.0	0.27-4.2
T4	HIGH	45	12-22
T3	HIGH	22	3.1-6.8

Increased Mortality RTH β



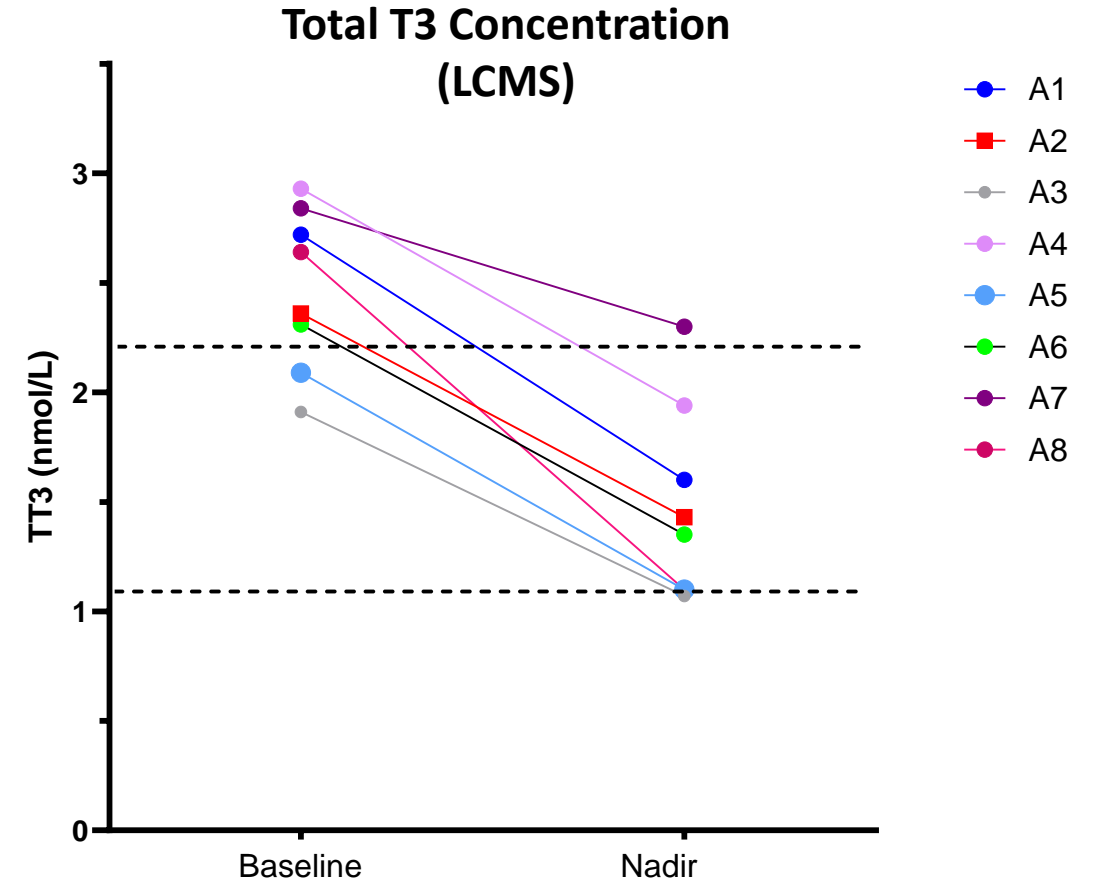
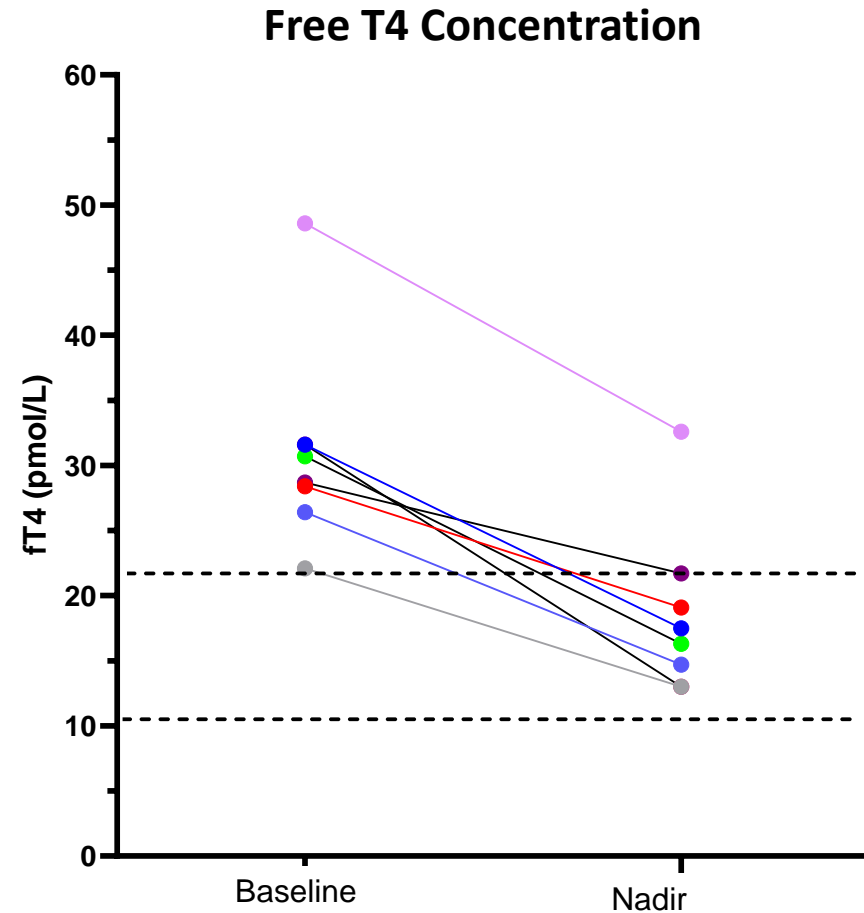
Welsh cohort

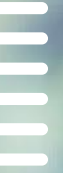
55 patients RTH Beta

2750 Age and sex matched controls

Median age 1st event 56 vs 67

Thyroid Hormone Concentration on Triac Treatment





4.

Financials

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

Announcement published on October 10, 2023



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



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



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

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

Announcement published on September 30, 2024

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



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

September 30, 2024

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

FDA granted Rare Pediatric Disease designation to Emcitate®

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



Overview of PRV

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

Examples of PRVs sold

Seller	Buyer	Value	Year
Rhythm Pharmaceuticals	Undisclosed	\$100M	2021
Albireo	Undisclosed	\$105M	2021
Biomarin	Undisclosed	\$110M	2022
BridgeBio	Undisclosed	\$110M	2022
Mallinckrodt	Novartis	\$100M	2022
Marinus Pharmaceuticals	Novo Nordisk	\$110M	2022
Ipsen	Undisclosed	\$158M	2024
PTC Therapeutics	Undisclosed	\$150M	2024

Share Register, Cash and Market Cap



10 Largest shareholders

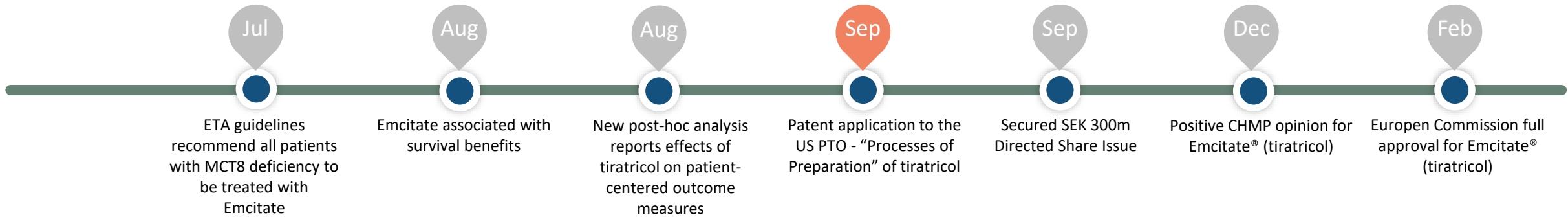
Name	Capital	Votes	EGTX	EGTX C	Verified
Frazier Life Sciences	13.22%	13.33%	38,675,501		2023-12-31
Cidro Förvaltning AB (Peter Lindell)	9.29%	10.04%	36,084,817		2025-01-29
Cetoros AB (Peder Walberg)	8.70%	9.40%	33,776,221		2025-01-29
Egetis Therapeutics AB	7.47%	0.81%		29,000,000	2025-01-29
Fjärde AP-fonden	6.94%	7.50%	26,942,859		2025-01-29
Avla Holding AB (Kennet Rooth)	4.55%	4.92%	17,668,330		2025-01-29
Unionen	3.55%	3.84%	13,800,000		2025-01-29
Handelsbanken Fonder	3.35%	3.62%	12,987,088		2025-01-31
RegulaPharm AB (Gudrun Hörnqvist)	2.71%	2.93%	10,531,660		2025-01-29
Avanza Pension	2.02%	2.18%	7,838,476		2025-01-29
Total 10	61.81%	58.57%	198,304,952	29,000,000	
Total number of owners	8,760				2025-03-04
Total number of shares	388,238,126				2025-03-04

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

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

* March 4, 2025

Egetis submits patent application to the USPTO



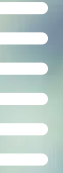
- Patent application for "Processes of Preparation" of tiratricol
- Processes and compounds described in the patent application
- If granted, this would be a significant patent for Egetis
- Generally, the exclusivity term of a new patent is 20 years from the date on which the application for the patent was filed in the United States.



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

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

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



5.

Summary

Egetis – a de-risked biotech with substantial unlocked potential



- Late stage biotech “under the radar”, developing the first therapy for a devastating genetic disorder
 - Strong team with established track record in the orphan drug space, including SOBI, Alexion, Biomarin, Biogen, Vertex, Sarepta, Shire and Wilson Therapeutics
- Strong data in clinical trials, demonstrating significant effects on key clinical outcomes
 - Supported by strong mechanistical rationale and data from animal models
- First approval for Emcitate for MCT8 deficiency in EU received in February 2025,
 - Already passed most of typical drug development risks
 - A small trial reconfirming the effect on biomarker T3 under way to complete the US NDA dossier
- Significant market opportunity with potential for premium orphan drug pricing
 - Disease awareness activities already bearing fruit
 - Continuous expansion of the Emcitate Managed Access Program confirms high unmet medical need
- Opportunity for indication expansion into RTH-beta
- Eligible for priority review voucher upon US approval, which can be sold for ~100-150 MUSD

Upcoming value enhancing key milestones 2025-2026



Emcite®

2025-2026

MCT8
deficiency

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

RTH-beta

- Potential initiation of Investigator Initiated Study - Egetis Industry collaborator

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 approved in EU in February 2025 for MCT8 deficiency
Pivotal trial for ***Emcitate*** NDA is ongoing

3

Highly attractive **orphan drug segment**

4

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

5

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

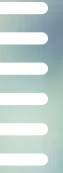
Listed on NASDAQ Stockholm (EGTX)

HQ in Stockholm, Sweden

~40 FTEs

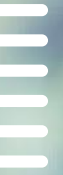


*In-house development of *Aladote* parked until *Emcitate* submissions have been completed



6.

Appendix



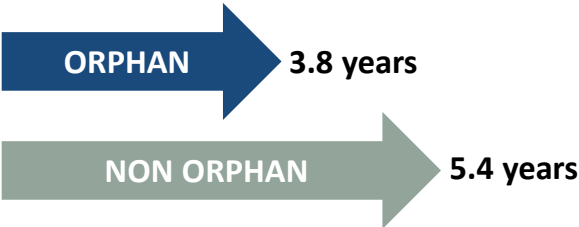
6.a

The attractiveness of the orphan drug segment

Orphan drug segment – a highly attractive opportunity

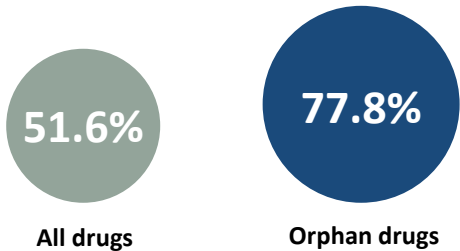
Shorter clinical development time¹

Phase II to launch Average # of years



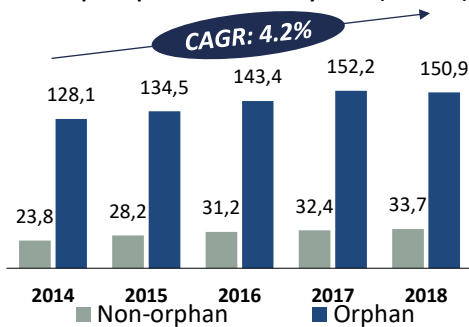
Higher probability of success³

Phase III to approval
POS in metabolic/endocrinology indications



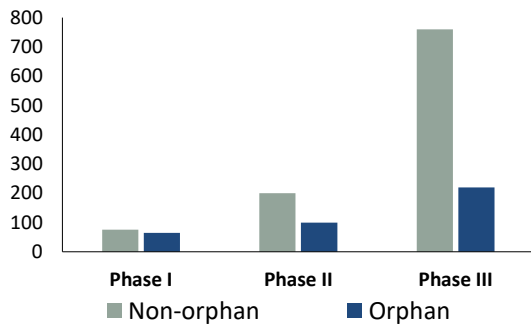
Higher attainable prices²

Mean cost per patient and year (USDk)

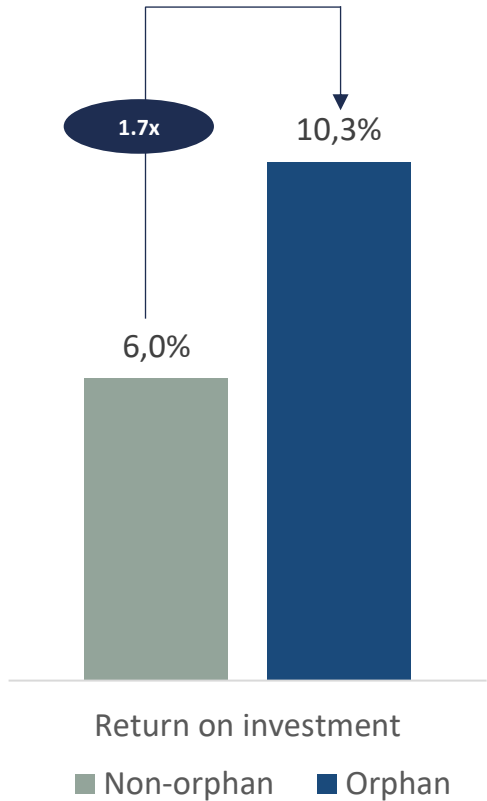


Fewer patients for clinical trials⁴

Patients per trial



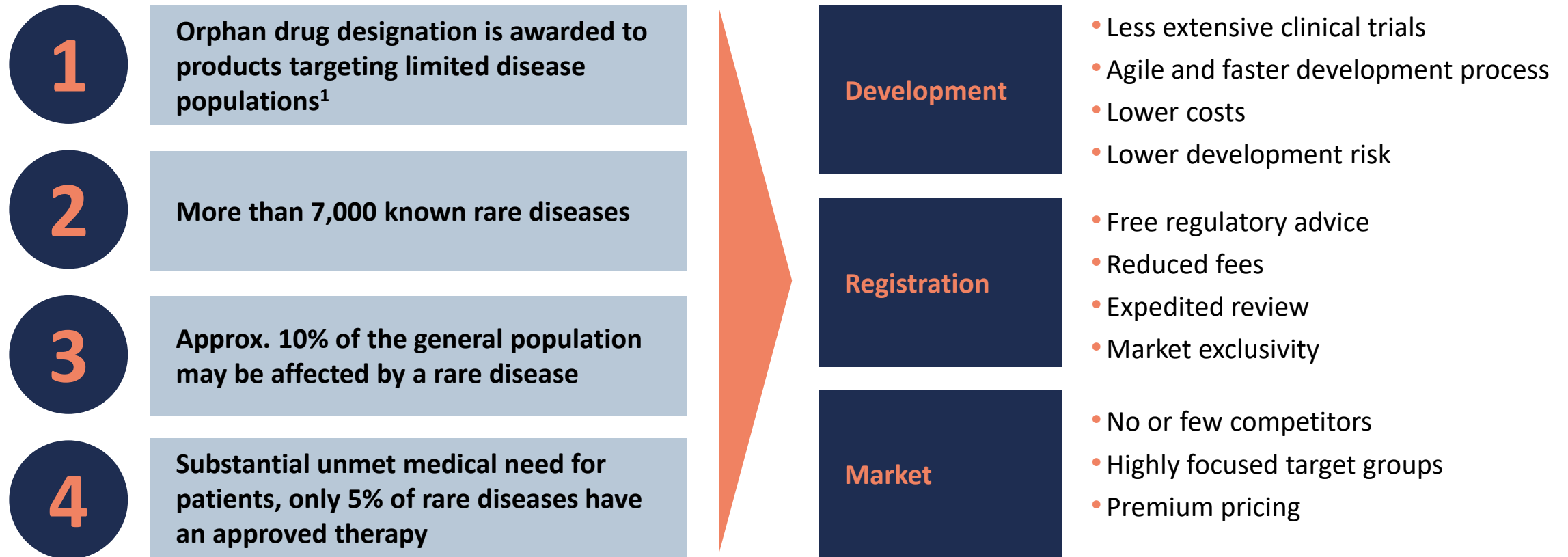
Orphan drugs attractive returns⁵



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

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

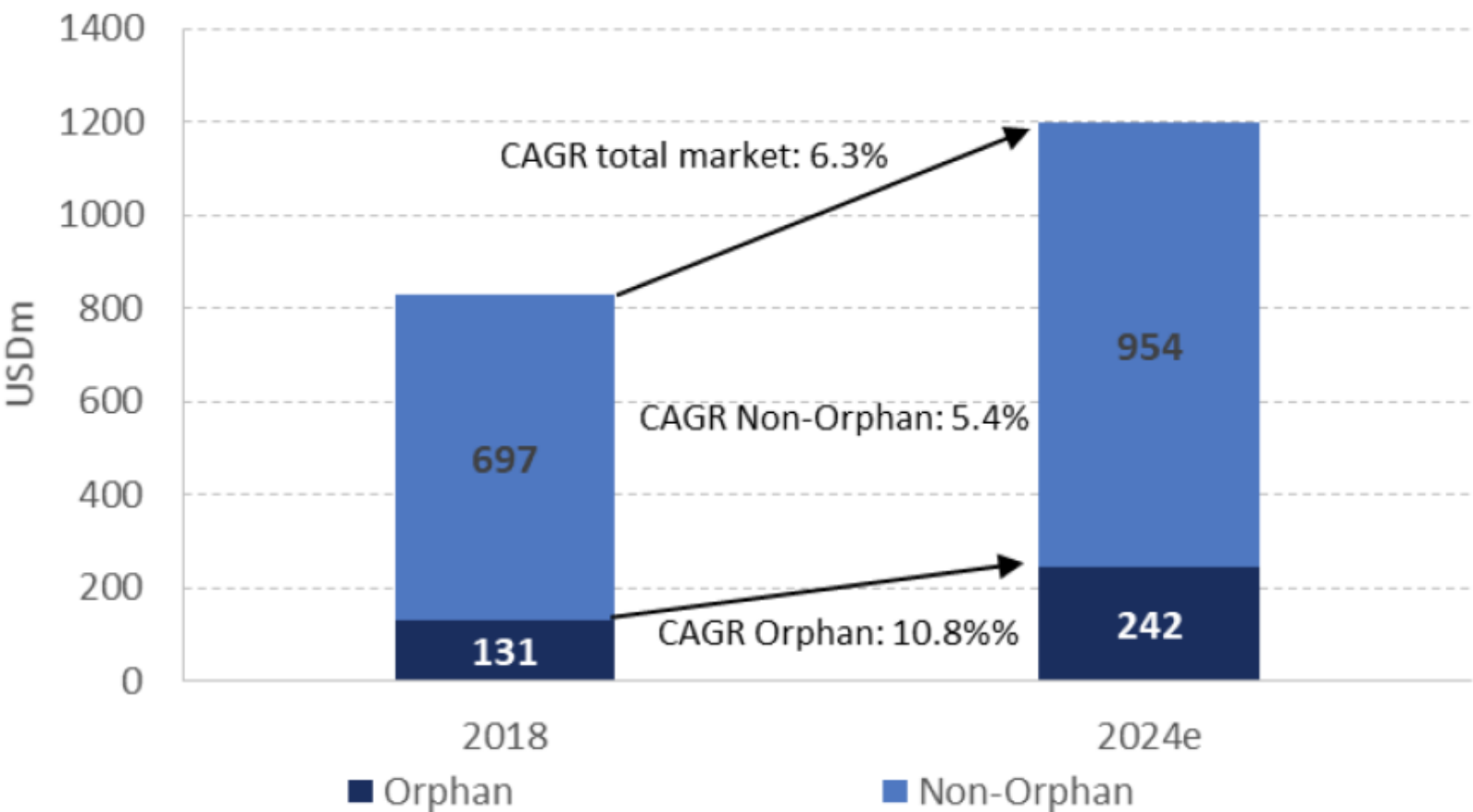
Orphan drug segment – a highly attractive opportunity

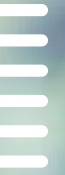


Well-defined patient populations with substantial unmet medical need

CAGR estimates of total pharmaceutical market vs orphan

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





6.b

Leadership Team and Board of Directors

Leadership team with global experience & proven track record



Nicklas Westerholm

CEO

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



Desiree Luthman

VP Regulatory Affairs

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



Katayoun Welin-Berger, PhD

VP Technical Operations

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



Yilmaz Mahshid, PhD

CFO

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



Kristina Sjöblom Nygren, MD

CMO

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



Anny Bedard

President Egetis North America

- Joined 2022
- Commercial leadership roles at Shire and Sarepta



Christian Sonesson, PhD

VP Product Strategy & Development

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



Henrik Krook, PhD

VP Commercial Operations

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



Nils Hallen

Global Head of HR

- Joined 2021
- Adjunct professor in work & organizational psychology



Laetitia Szaller

General Counsel & Head of Compliance

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



Karl Hård, PhD

VP IR & Business Development

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

Board of directors



Mats Blom

Chair of the board since 2024

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



Gunilla Osswald

Board member since 2017

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



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



Margarida Duarte

Board member since 2025

- Shares in Egetis: 0
- BS in Pharmaceutical Sciences & Executive Master's degree in Medical Marketing Management
- Other assignments: Executive Vice President, Global Chief Commercial Officer, Deciphera Pharmaceuticals



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

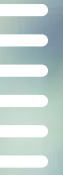
Termination of discussions regarding a potential acquisition of the Company



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. Positive CHMP opinion received in December 2024.



6.c

Paracetamol/Acetaminophen overdose and clinical experience with Aladote

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

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



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

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

Paracetamol/acetaminophen poisoning

– *no adequate treatment for increased-risk patients*



What is
paracetamol/
acetaminophen
poisoning?

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

How many does
it affect?

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

Why is current
treatment
inadequate?

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

A new standard
of care is
needed

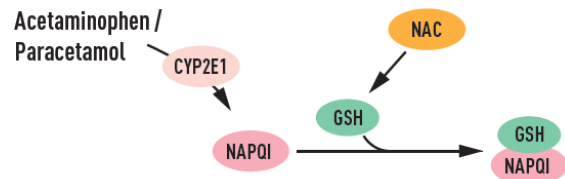
- **Aladote®** aims to become **a new standard of care** for patients with increased risk for liver injury in combination with NAC

Orphan drug candidate

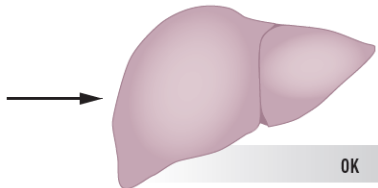
with clear scientific and mechanistic rationale

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

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

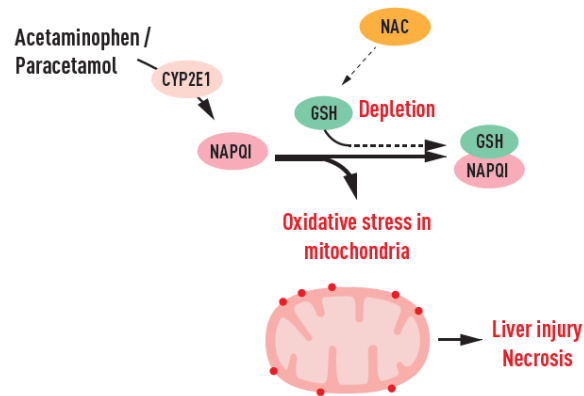


- In most cases NAC effectively prevents liver injury i.e. limited need for Aladote®

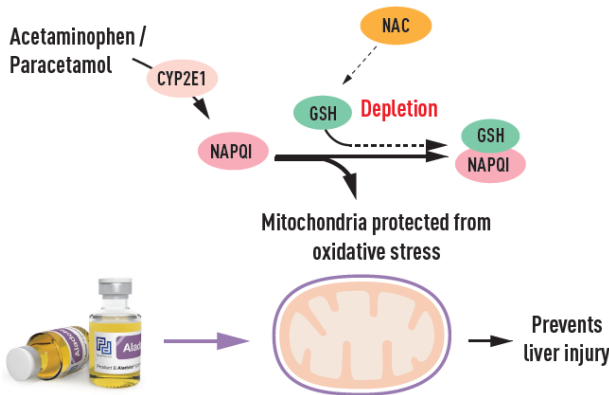


Late presenters (>8h) are at increased-risk for liver injury NAC treatment + Aladote® to prevent liver injury

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



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



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

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

Overview of completed Phase Ib/IIa

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



Positive proof-of-principle Phase Ib/IIa results

Indicates that Aladote may reduce liver injury

Safety & tolerability

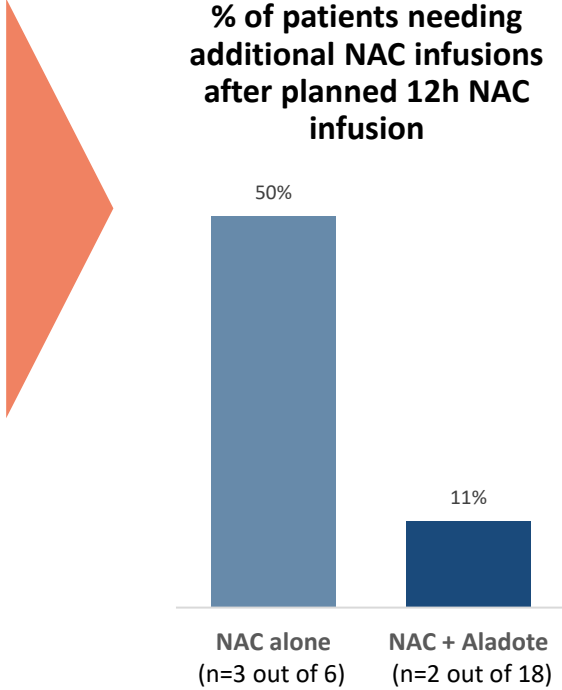
Event	NAC alone	NAC + 2 μmol/kg Aladote	NAC + 5 μmol/kg Aladote	NAC + 10 μmol/kg Aladote
Any AE	6 (100%)	6 (100%)	6 (100%)	6 (100%)
Any SAE	2 (33%)	4 (67%)	2 (33%)	3 (50%)
SAE Starting within 7 days	1 (17%)	1 (17%)	1 (17%)	2 (33%)

- 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 – ALT¹ 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

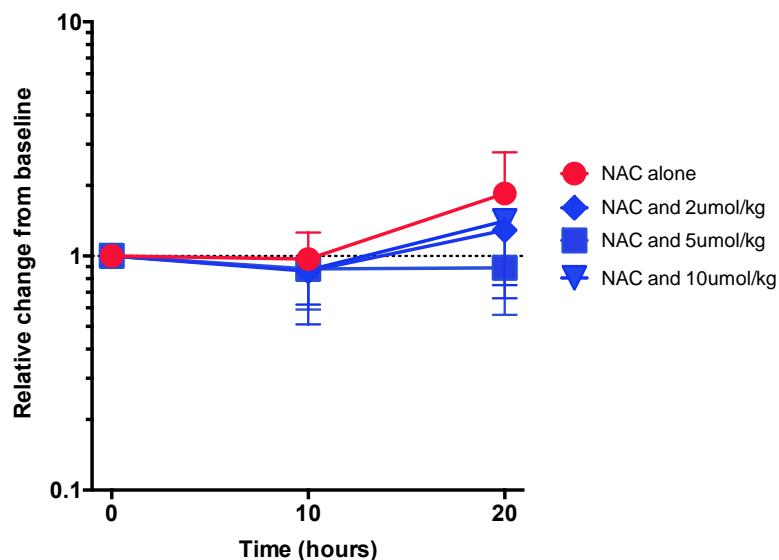


Note: (1) Alanine transaminase (ALT) is a transaminase enzyme found in plasma and in various body tissues especially the liver’s hepatocytes. Serum ALT is commonly measured clinically as part of a diagnostic evaluation of hepatocellular injury, to determine liver health

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

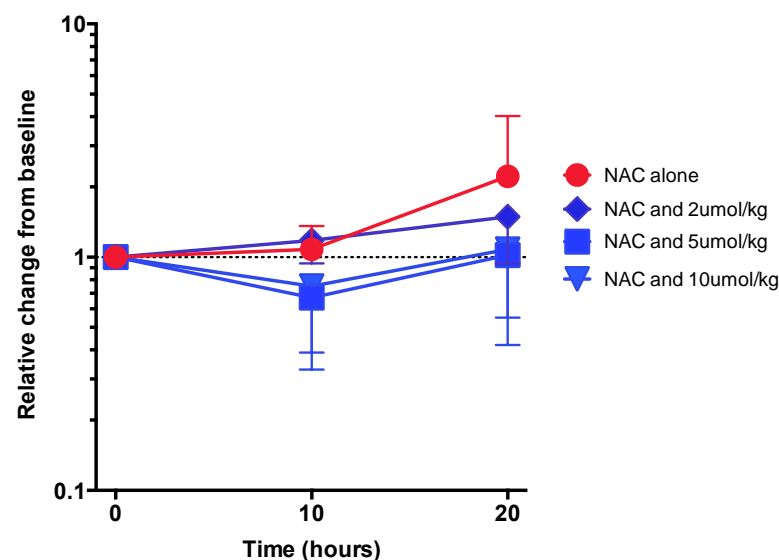


K18



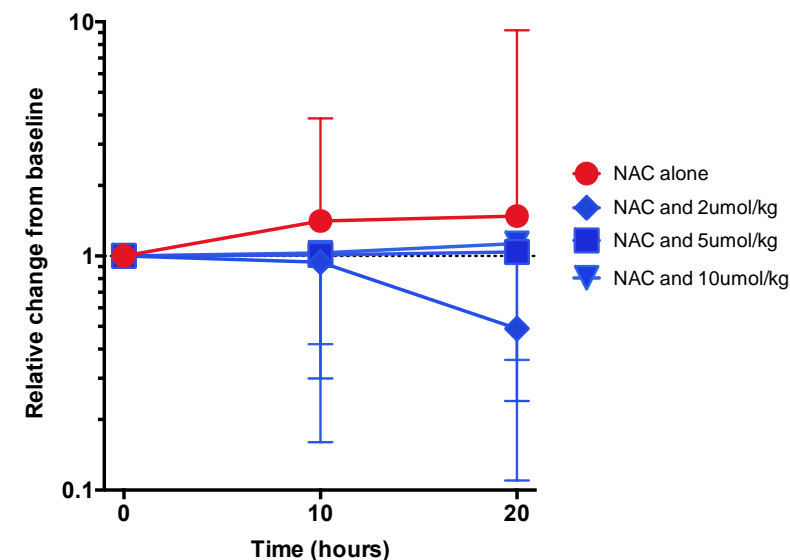
K18 is a measure of cell death and correlate with peak ALT activity during the hospital stay

ccK18

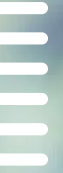


ccK18, is a measure of cell death and correlate with peak ALT activity during the hospital stay

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* MCT8 deficiency submissions have been completed

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



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

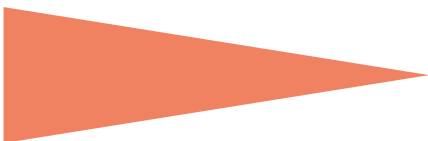


*Study parked until *Emcitate* submissions have been completed

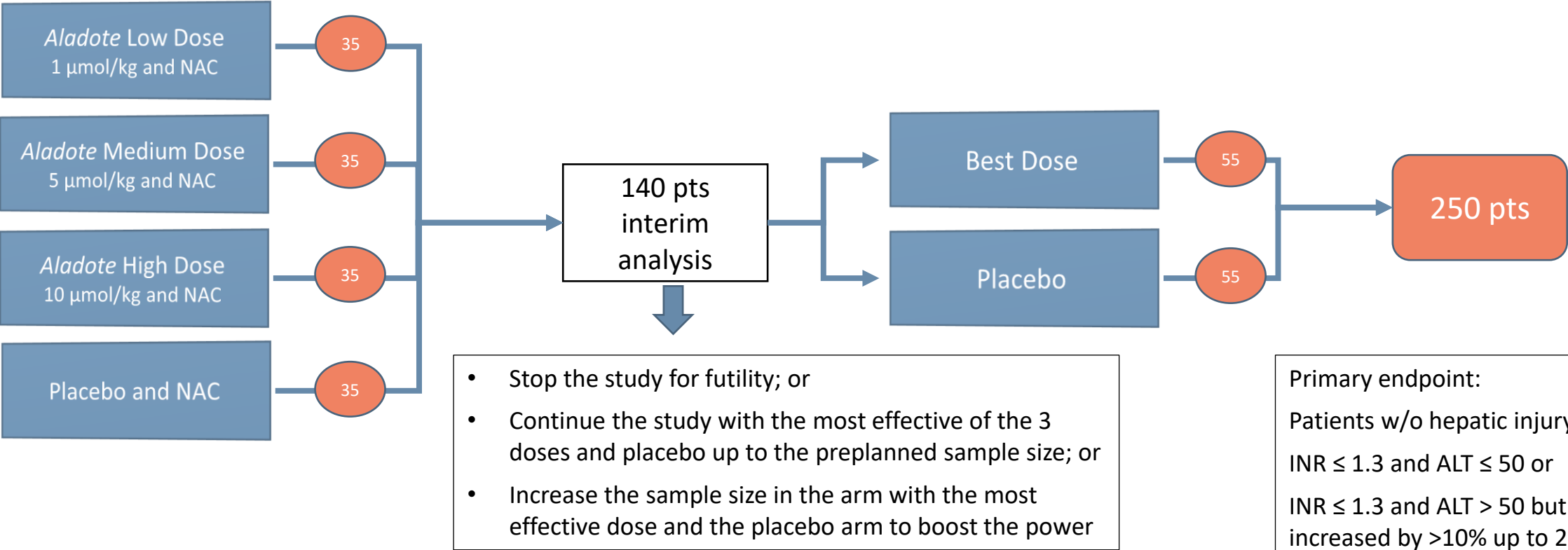
ALBATROSS: Aladote Phase IIb/III study design



Seamless Phase IIb/III design



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



Aladote clinical development timelines

✓ Orphan Drug Designation EU

✓ CTA pivotal Phase IIb/III study

2022

tbc

tbc

tbc

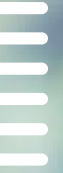
- Start pivotal Phase IIb/III study (after *Emcitate* submissions have been completed)

- Interim analysis
- Recruitment completed and topline results

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

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





3.

Aladote® - Commercial opportunity

Aladote– alleviating patient and societal burden

Aiming to provide value for both patients and society



POD is a life threatening condition with remaining medical needs

Patients

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

Society

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



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

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

Commercialisation of *Aladote* for high-risk POD patients

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



Favorable conditions for launch success

Addressing unmet medical need



Leading KOL support



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



Treatment choice **highly protocol driven**



No competition

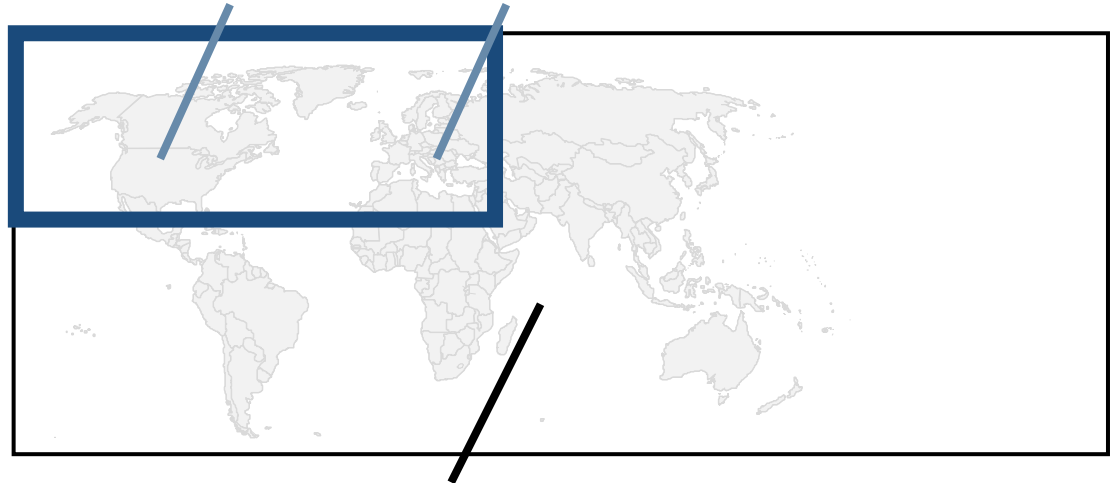


Addressing life-threatening condition

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

Hospitalized POD patients per year

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



Commercialization in rest of world managed through partners

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

Analogue antidotes priced at \$ 3.5k – 50k

National emergency hospital stocking guidelines - opportunity for rapid market penetration

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

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

Thank you!

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