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ØU Life Science Seminar, May 31, 2023

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

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Agenda

1. An integrated orphan drug company, focusing on late-stage development for commercialization
    - Pipeline overview
2. Emcitate® for treatment of MCT8-deficiency
    - Overview of MCT8-deficiency
    - Emcitate and review of clinical data to date
    - Regulatory pathway to submissions in EU and US
    - Disease awareness and commercial opportunity
3. Summary

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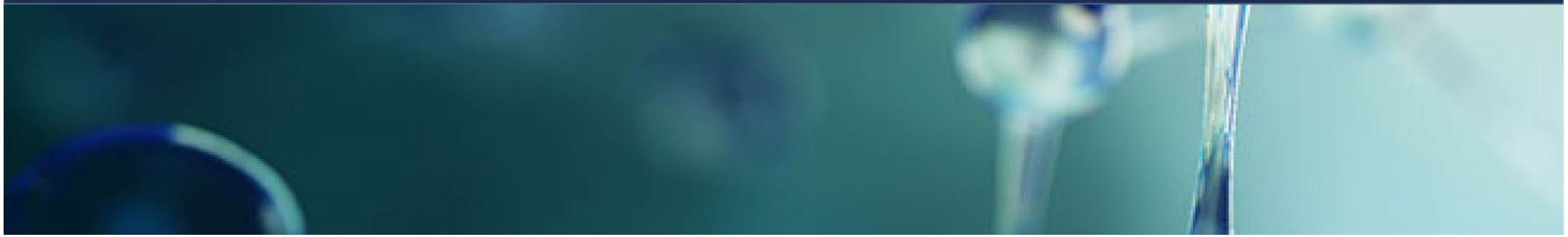
1.

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



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

1. Dedicated orphan drug company
   Two late-stage assets: ***Emcitate*** and ***Aladote***
2. Target **MAA/NDA** submissions:
   Emcitate **2023** and *Aladote* **2025**
3. Highly attractive **orphan drug segment** with potential **>$1Bn annual sales opportunity**
4. Plan to **launch** through **small in-house commercial** organization in the EU and North America
5. **Strong team** with late-stage orphan clinical development, registration and commercialization experience from:

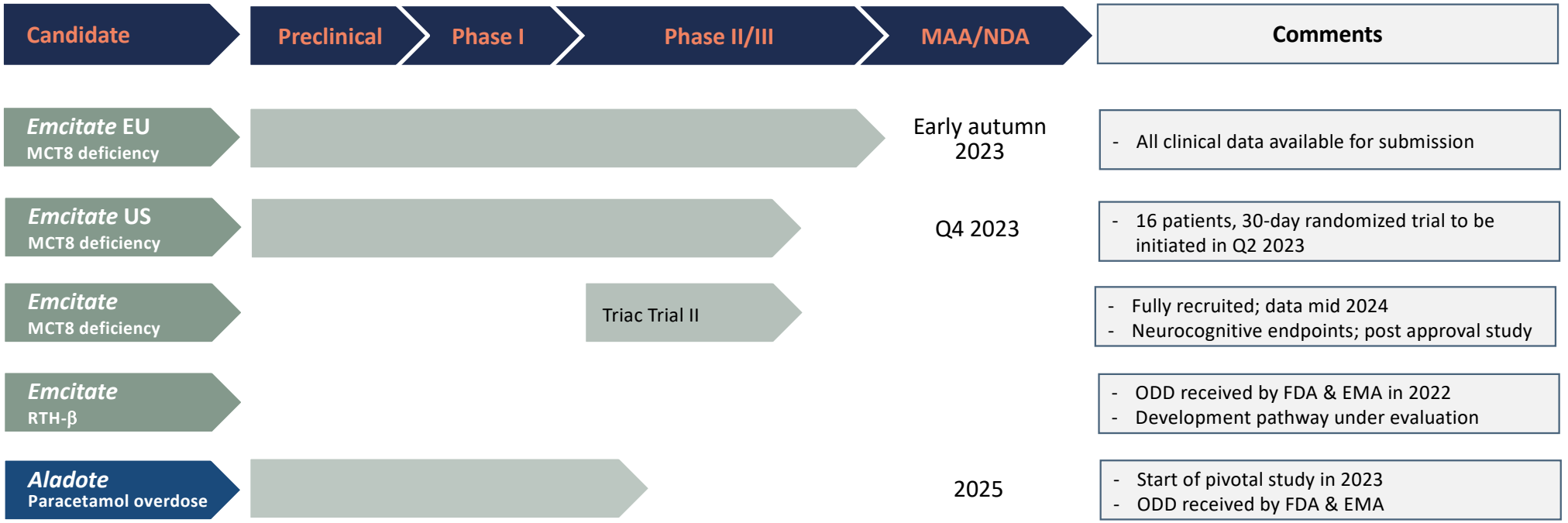
Listed on NASDAQ Stockholm (EGTX)

HQ in Stockholm, Sweden

~30 FTEs

Pipeline overview

Planned Emcitate EU and US filings in 2023



2.

Overview of MCT8-deficiency

Overview - MCT8 deficiency

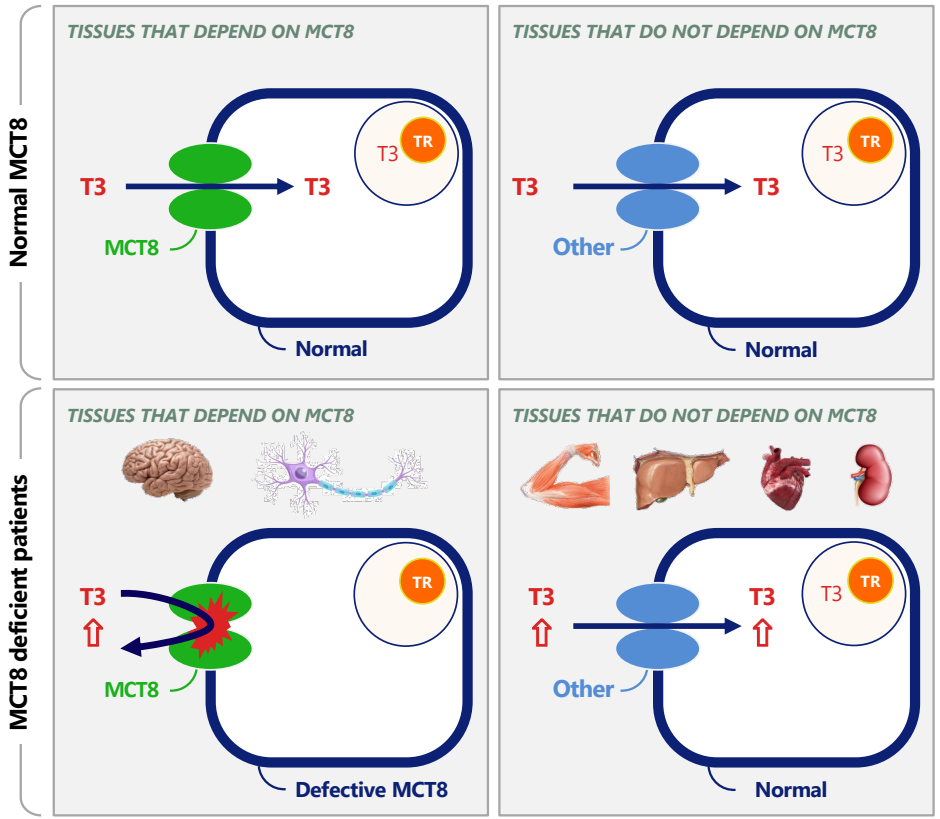
CAUSE
- Mutation in the gene for thyroid hormone transporter MCT8
- X-linked

INCIDENCE
PREVALENCE
- 1: 70,000 boys
- 10-15,000 cases globally[1]

SYMPTOMS
- Severe intellectual disability (cerebral hypothyroidism)
- Peripheral thyrotoxicosis
- Median life expectancy - 35 years

TREATMENTS
- No available therapy

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



1. Includes US, EU and RoW approachable population (e.g. UK, Australia, Canada, Japan, Switzerland, South Korea and Turkey)

MCT8 Deficiency - Debilitating Disease Impacting Mortality and Quality of Life

Peripheral thyrotoxicosis
Low body weight
Long term cardiovascular issues
Decreased muscle mass
Hypertension
Hypermetabolism
Sleep disturbances
Malnutrition

Quick facts from natural history	
Median onset of symptoms:	4 months
Median age of diagnosis:	~1 year
Severe intellectual disability:	100%
Ability to sit independently:	8%
Hypotonia, hypertonia & primitive reflexes:	90%
Cardiac arrythmias (PAC):	76%
Median life expectancy:	35 years

Cerebral hypothyroidism
Severe intellectual & motor disability
Limited ability to sit, stand, walk
Limited ability to communicate
Life-long 24-hour care

2.

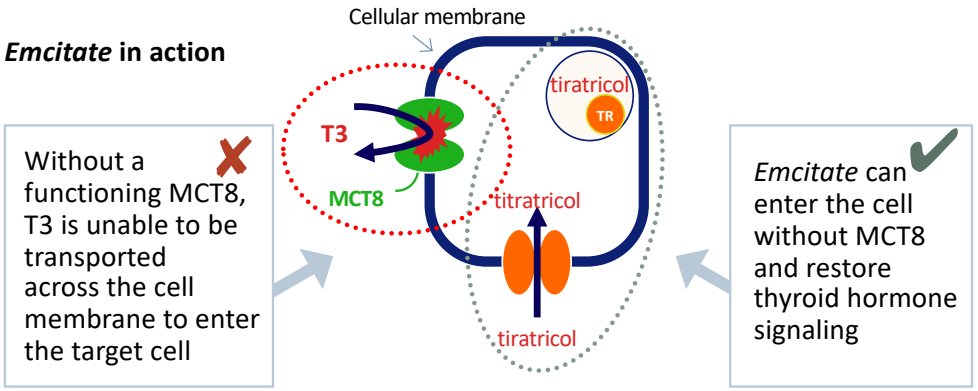
Clinical experience with Emcitate

Orphan drug candidate

with clear scientific and mechanistic rationale and established safety profile

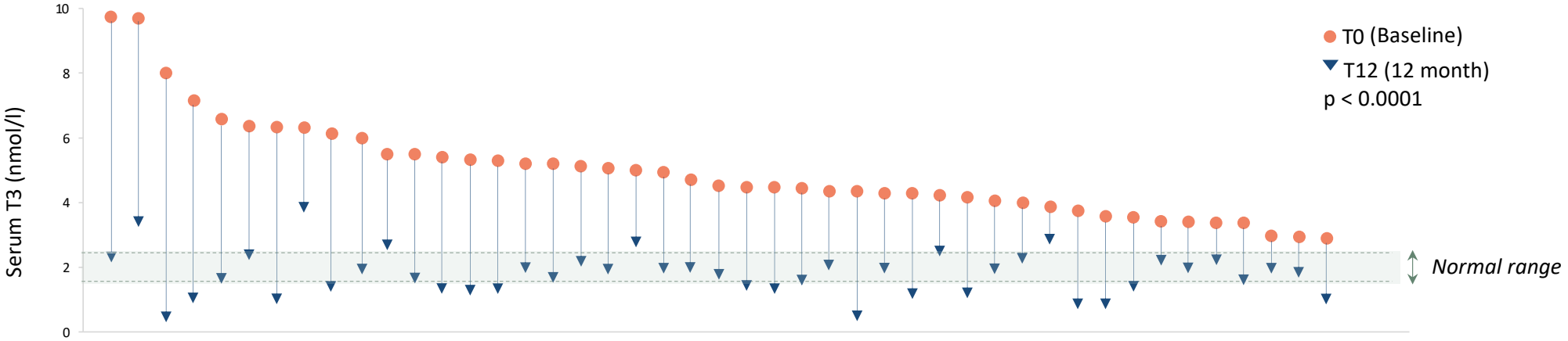
***Emcitate* (tiratricol) – Addressing MCT8 deficiency**

- 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



Consistent, clinically relevant and highly significant results

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

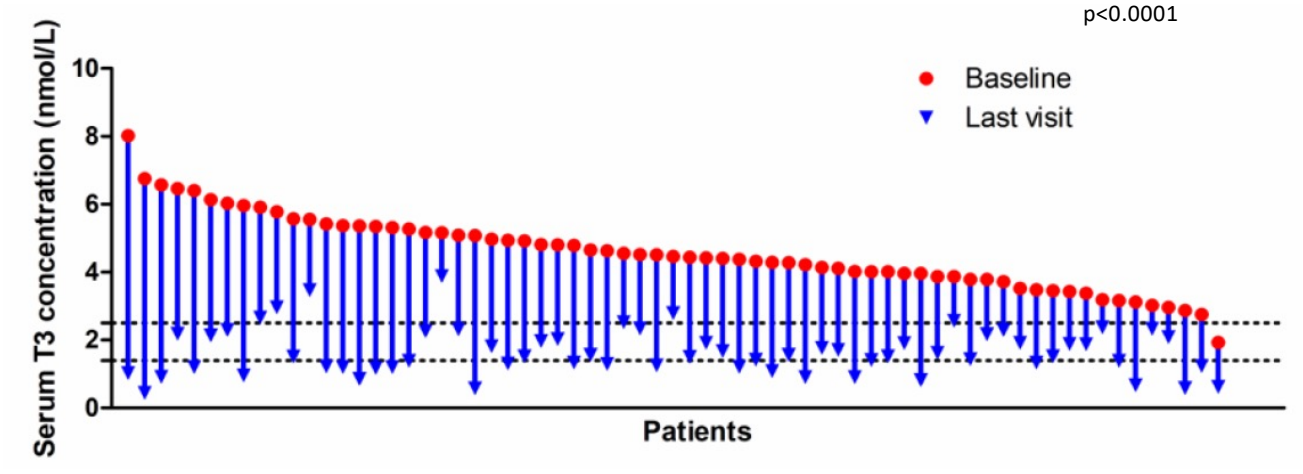


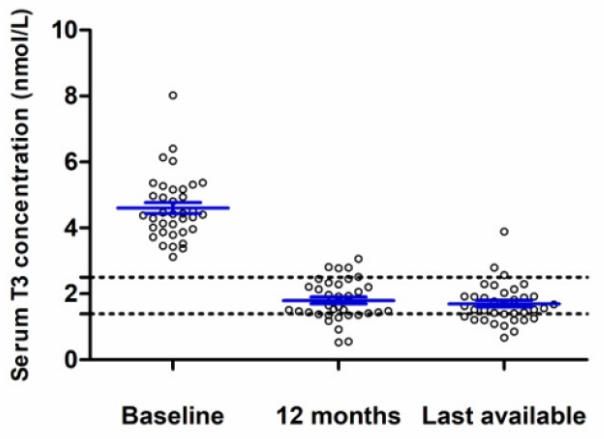
Endpoints	Baseline mean *(± SD)*	12 months mean *(± SD)*	Difference in means *(95% CI)*	p-value
Serum T3 (nmol/L)	4.97 (± 1.55)	1.82 (± 0.69)	-3.15 (-3.62, -2.68)	<0.0001
Weight to age (z score)	-2.98 *(± 1.93)*	-2.71 *(± 1.79)*	0.27 *(0.03, 0.50)*	0.025
Resting heart rate (bpm)	112 *(± 23)*	104 *(±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

Source: Groeneweg et al; Lancet D&E 2019

New cohort confirms primary endpoint results in Triac Trial I

Fast and durable normalization of T3 values in almost all patients





2.

Emcitate® - regulatory pathway to submissions in EU and US

Regulatory features of *Emcitate* for MCT8 deficiency

ODD

Orphan drug designation for MCT8 deficiency
Eligibility: Market exclusivity 10y (EU) & 7y (US)

Fast track

Fast track designation (FDA)
Eligibility: Six months review of NDA & rolling submission

PRV

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

MAA NDA

MAA: All clinical data available (submission early autumn ’23)
NDA: Small confirmatory study agreed with FDA (submission Q4-’23)

ODD

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

*The voucher may be sold to another sponsor (2021-22 range: $100m-$110m)

Emcitate regulatory pathway to submissions in EU and US

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

Included in MAA in EU in early autumn 2023

Included in NDA in US Q4 2023 under the Fast Track Designation

To be added post approval when data available

Triac Trial I

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

EMC cohort study

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

Natural history

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

Controlled study (ReTRIACt)

- Start in Q2 2023
- N= 16
- Pts from named patient/ compassionate use program
- Top line results Q4 2023

Triac Trial II

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

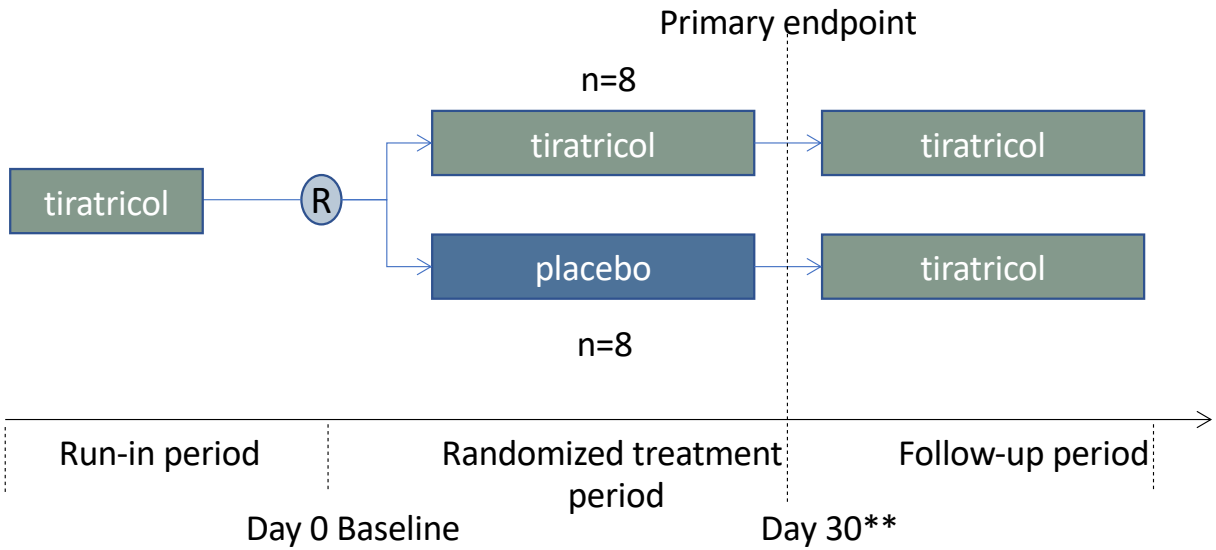
Data already available

ReTRIACt – Measuring proportion of patients meeting T3 ≥ULN within the randomized treatment period

Randomised placebo controlled study to verify previous single arm Triac I trial and real-world cohort study results

- FDA acknowledges that a treatment effect on T3 levels and the manifestations of chronic thyrotoxicosis in MCT8-deficiency could provide a basis **for marketing approval** also in the US.
- A small, 30-day, placebo-controlled study in 16 treated patients, to be identified primarily through our existing named patient program, will be conducted to **verify the results on T3** levels seen in previous clinical trials and publications in a randomized **controlled** setting.
- An **NDA** in the US is targeted to be submitted in **Q4 2023** under the Fast Track Designation.
- A major step towards marketing authorization and increases the likelihood of success for *Emcitate* and the probability to receive a US Rare Pediatric Disease **Priority Review Voucher (PRV)**.

Controlled Study (ReTRIACt) – design agreed with FDA



Primary endpoint: Proportion of participants who meet the rescue criterion (T3>ULN) during the 30-day double-blind Randomized Treatment Period

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

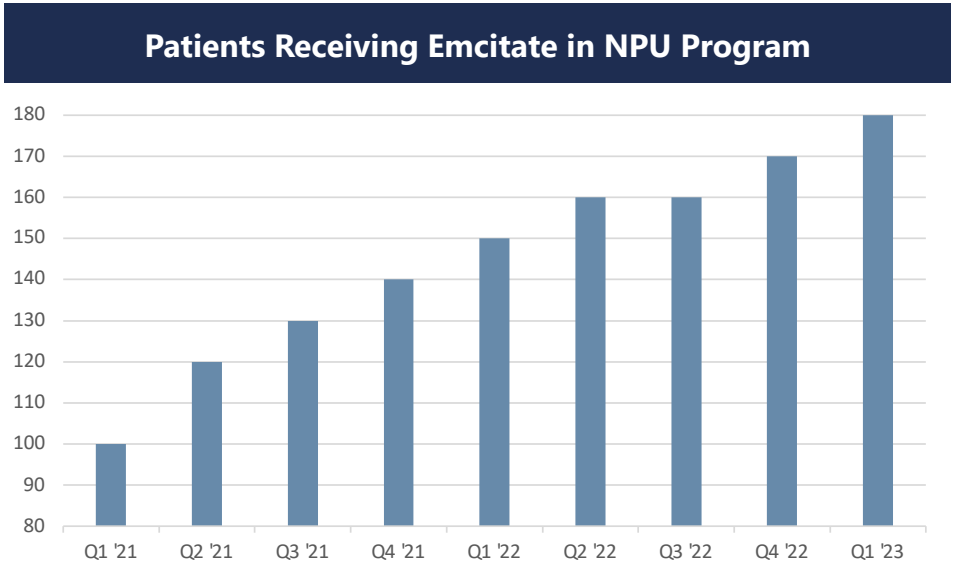
2.

Emcitate® - Commercial opportunity

Emcitate supplied globally on a named patient basis

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

- NPU and compassionate use programs
  - mechanisms to allow early access to a medicine prior to regulatory marketing approval
  - granted to pharmaceuticals under development for situations with high unmet medical needs and where no available treatment alternatives exist or are suitable
- Implemented Expanded Access Program as requested by the FDA - will Simplify Process for Accessing Emcitate
- Emcitate is being supplied on a named patient basis, following individual approval from the national medicines agencies, to
  - around 180 patients
  - in over 25 countries



Commercialization of *Emcitate*

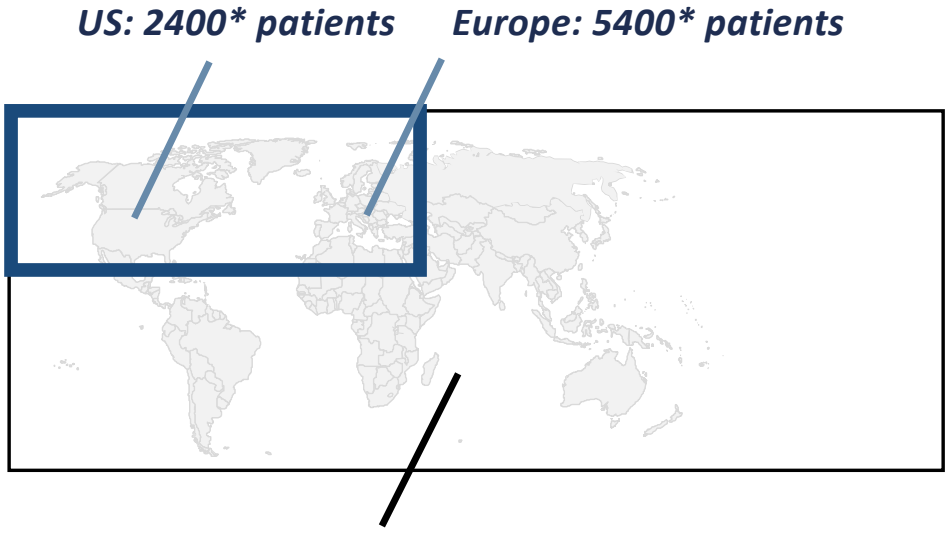
Disease area conditions provide opportunity for lean commercialization

Favorable conditions for launch success

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

Stepwise establishing inhouse commercial capabilities

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

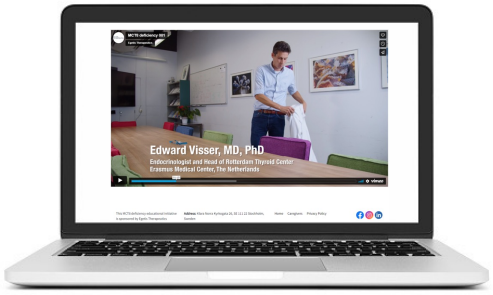


*Based on prevalence 1:70,000 males

Enabling patient identification through disease awareness

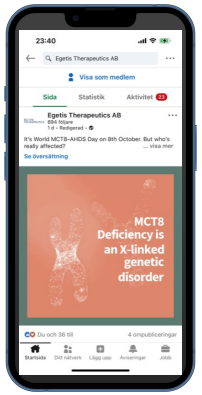
MCT8 deficiency awareness and educational activities launched through various channels

mct8deficiency.com



MCT8 - AHDS Foundation

Ultra-rare disorder
Striving for a cure. Empowering families



DISEASE AWARENESS AND EDUCATION

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

COLLABORATION WITH PAGs & KOLs

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

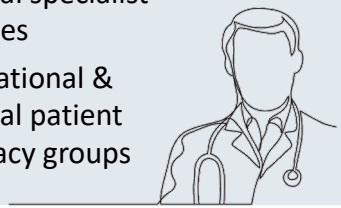


EXHIBIT AT SCIENTIFIC/MEDICAL CONFERENCES

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

OPTIMUM CHANNEL MIX FOR MAXIMUM REACH

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

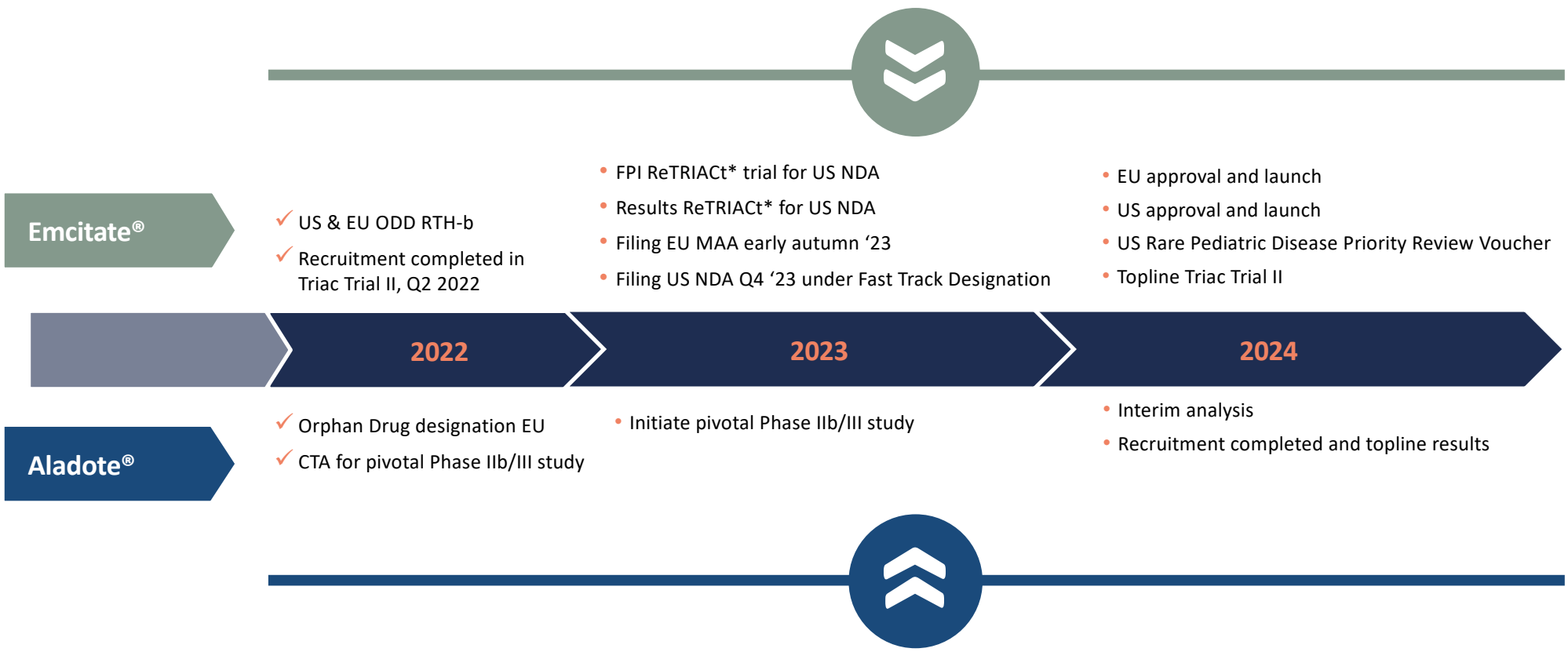
3.

Summary

EGTX – a de-risked biotech with substantial unlocked potential

- Late stage biotech “under the radar”, developing the first therapy for a devastating genetic disorder
  - Strong team with established track record in the orphan drug space, including SOBI, Alexion, Biomarin, Biogen, Vertex, Sarepta, Shire and Wilson Therapeutics
- Strong and consistent data in clinical trials, demonstrating significant effects on key clinical outcomes
  - Supported by strong mechanistical rationale and data from animal models
- High likelihood to reach market in 2024, already passed most of typical drug development risks
  - All clinical data necessary for regulatory approval in EU already at hand – Submission Early autumn 2023
  - A small and short trial reconfirming the effect on biomarker T3 under way to complete the US dossier - Submission Q4 2023
- Significant market opportunity with potential for premium orphan drug pricing
  - Estimated 2,400 affected patients in US and 5,400 in Europe
- Eligible for priority review voucher upon US approval, which can be sold for ~100 MUSD

Upcoming pipeline milestones



* 16 pts randomized 30 day study for US NDA

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Thank you!

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