



Corporate presentation

A new specialised late-stage orphan drug development company

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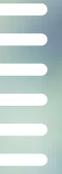
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Agenda



1. A new specialised late-stage orphan drug development company
2. Emcitate[®]
 - Clinical development programme
 - Commercial opportunity
3. Aladote[®]
 - Clinical development programme
 - Commercial opportunity
4. The Orphan drug segment and path to market
5. Summary
- A. Appendix



1.

A new specialised late-stage orphan drug development company

New specialised late-stage orphan drug development company



- 1** Dedicated orphan drug development company with two late-stage orphan drug assets: **Emcitate[®]** and **Aladote[®]**
- 2** Highly attractive **orphan drug segment** with potential **>\$1Bn annual sales opportunity**
- 3** Clear path to **market approval in EU and US** within **3 years**
- 4** Plan to **launch** through niche inhouse commercial organization in the EU and US
- 5** Combined core expertise in **late-stage orphan clinical development, registration and commercialization** with experience from:



The combination will drive synergies

PledPharma and Rare Thyroid Therapeutics merged to launch a new company



PledPharma

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

Rare Thyroid Therapeutics

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

Synergistic orphan drug focus

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

The acquisition and rights issue

Institutional investor base broadened



Acquisition

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

Rights issue

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

Orphan drug segment – a highly attractive opportunity



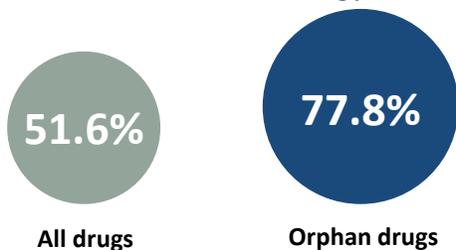
Shorter clinical development time¹

Phase II to launch Average # of years



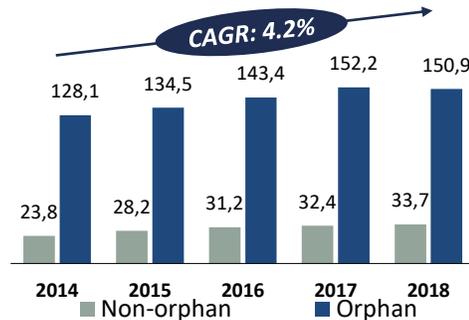
Higher probability of success³

Phase III to approval
POS in metabolic/endocrinology indications



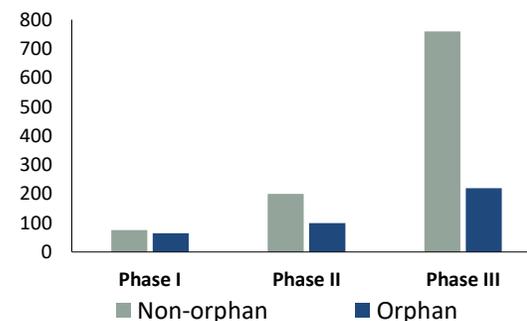
Higher attainable prices²

Mean cost per patient and year (USDk)

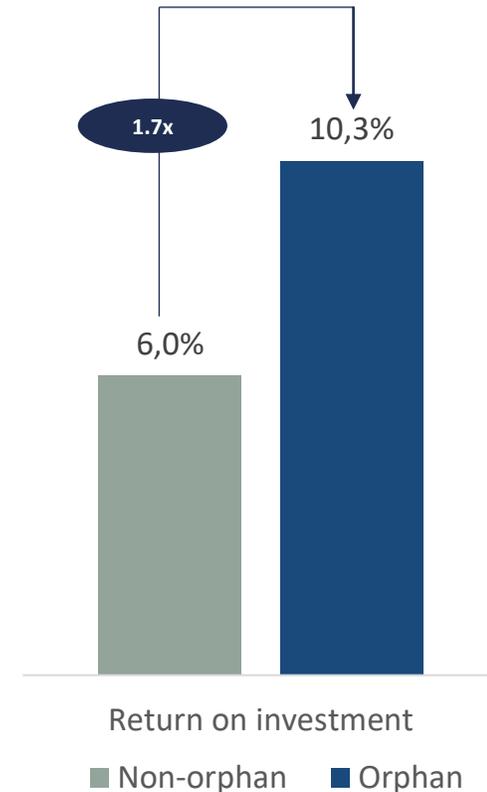


Fewer patients for clinical trials⁴

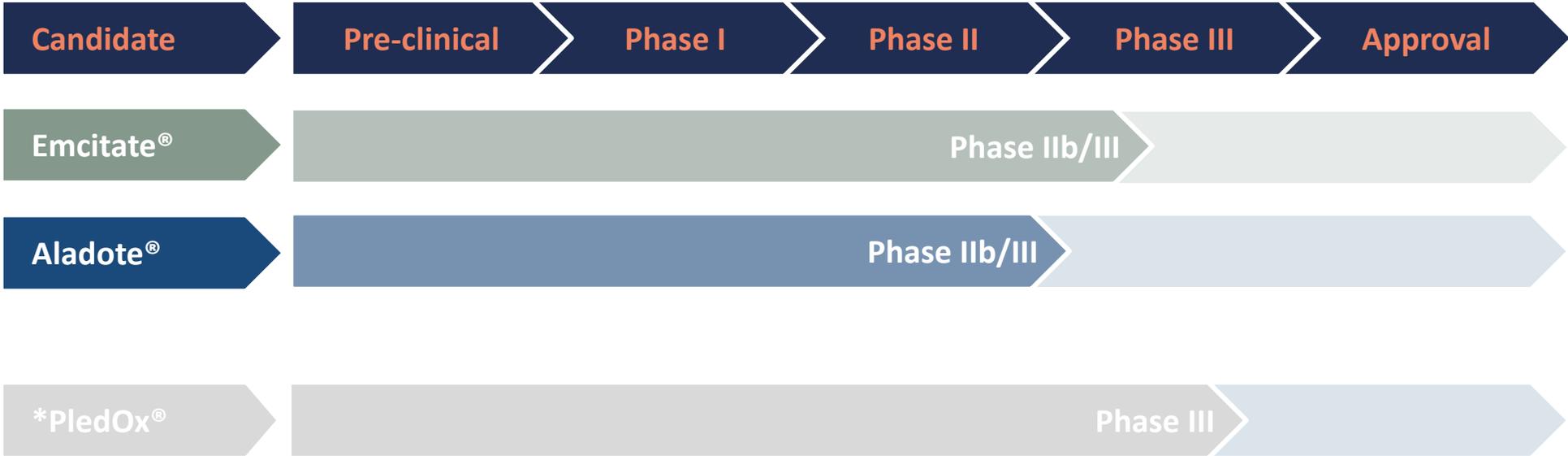
Patients per trial



Orphan drugs attractive returns⁵



Late-stage orphan drug pipeline addressing billion dollar markets



↑

* PledOx[®] did not meet the efficacy endpoint in the prematurely closed Phase 3 POLAR program. Based on further evaluation of the results from the POLAR studies, the strategic next steps for PledOx[®] will be determined together with Solasia.

Two highly promising orphan drug candidates

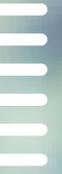


Emcitate® – Therapy for genetic disturbance in thyroid hormone signalling with life-long severe disability

- Lead candidate for addressing MCT8 deficiency, a condition with high unmet medical need and no available treatment
- Rare disease which affects ~1:70,000 males
- Obtained Orphan drug designation in the EU and US 2017 and 2019 respectively. **US Rare Paediatric Disease Designation received in November 2020**, eligible for Priority Review Voucher.
- Phase IIb clinical trial completed with significant and clinically relevant effects
- Pivotal Phase IIb/III early intervention trial in young subjects initiated with **first patient dosed in Dec 2020**
- No competing products in clinical development

Aladote® – Prevents acute liver injury caused by paracetamol poisoning

- Paracetamol poisoning is one of the most common overdose with >175,000 hospital admissions globally per annum
- No adequate treatment for increased risk patients exists
- Orphan drug designation (ODD) granted in 2019 in the US
- Eligible for ODD in the EU as a result of Brexit, application planned for Q1 2021
- Successful results from Phase Ib/IIa study in paracetamol overdosed patients
- Pivotal Phase IIb/III study planned for marketing authorisation application in both US and EU, ongoing interactions with the regulatory agencies (FDA, EMA and MHRA) to finalize study specific details
- No competing products in clinical development



2.

Emcitate[®] - clinical development programme

MCT8 deficiency: a detrimental condition with significant unmet medical need



What is MCT8 deficiency?

- Genetic disorder resulting in impaired thyroid hormone trafficking across cellular membranes
- MCT8 is one of the key thyroid hormone transporters in the body
- Mutation located to the X chromosome, affecting only males
- Estimated prevalence of 1:70,000 males



Patients with MCT8 Deficiency¹⁾

What does it mean?

- Absence of a functional MCT8 protein means that thyroid hormone is not able to pass into cells dependent on MCT8 and importantly cross the blood-brain-barrier, resulting in too low or no thyroid hormone levels in such tissues, including the brain
- Disrupted feedback loop mechanism results in a compensatory increase in circulating thyroid hormone
- Tissues depending on other transporters than MCT8 for thyroid hormone transport will suffer from too high thyroid hormone levels
- Simultaneous too high and too low thyroid hormone stimulation in different tissues

What are the challenges?

- Patients appear normal at birth with normal weight, length and head circumference with no evident signs of significant thyroid hormone disturbance
- Initial symptoms appear within the first months of life
- Disruption of normal neurodevelopment in childhood resulting in severe intellectual disability.
- Most patients never develop autonomy or ability to sit or walk and have limited ability to communicate
- Life-long morbidity from disturbed thyroid hormone pattern, resulting in agitation, cardiovascular symptoms, wasting and impaired life expectancy
- Heavily dependant on caregivers resulting in very high disease burden

How do you manage the disease?

- Currently no therapy available to address the root cause of the disorder
 - Standard therapeutic approaches for thyroid dysfunction not effective or suitable
 - Easy diagnosis once considered with readily available, low-cost laboratory test
 - Large proportion of patients remain undiagnosed with significant delay to diagnosis
- ▼
- Significant unmet medical need from a humanitarian, health economic and societal perspective

Quick facts from natural history²

Median life expectancy:	35 years
Median onset of symptoms:	4 months
Median age of diagnosis:	24 months
Patients surviving into adulthood:	70%
Severe intellectual disability:	100%
Global delay in myelination:	100%
Reduced white matter volume:	100%
Neurocognitive development age:	<12m
Ability to sit independently:	8%
Global hypotonia, hypertonia and persistence of primitive reflexes:	90%
Requires tube feeding:	36%
Severe underweight:	75%
Cardiac arrhythmias (PAC):	76%
Life long 24-hour care:	100%

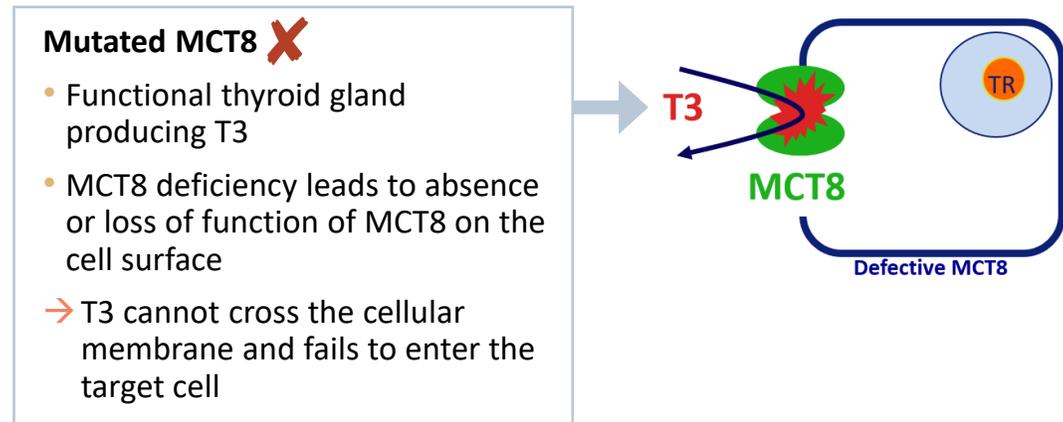
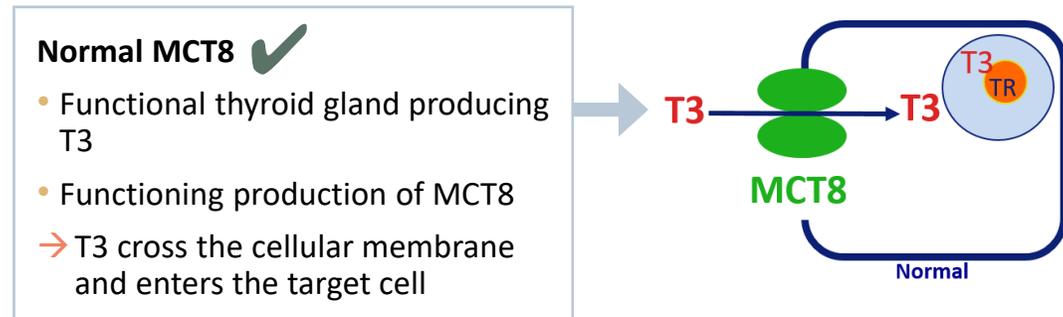
Orphan drug candidate

with clear scientific and mechanistic rationale and established safety profile



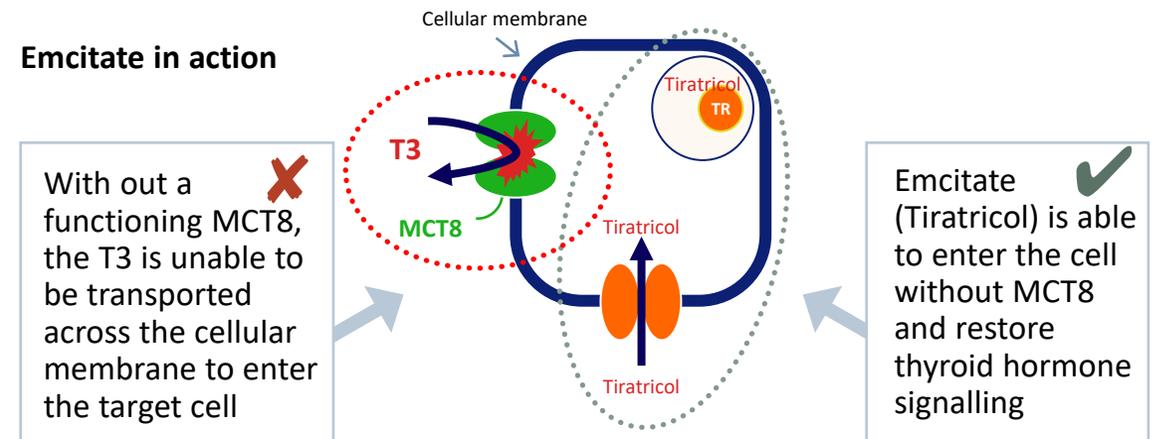
Difference normal MCT8 and deficiency of MCT8

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

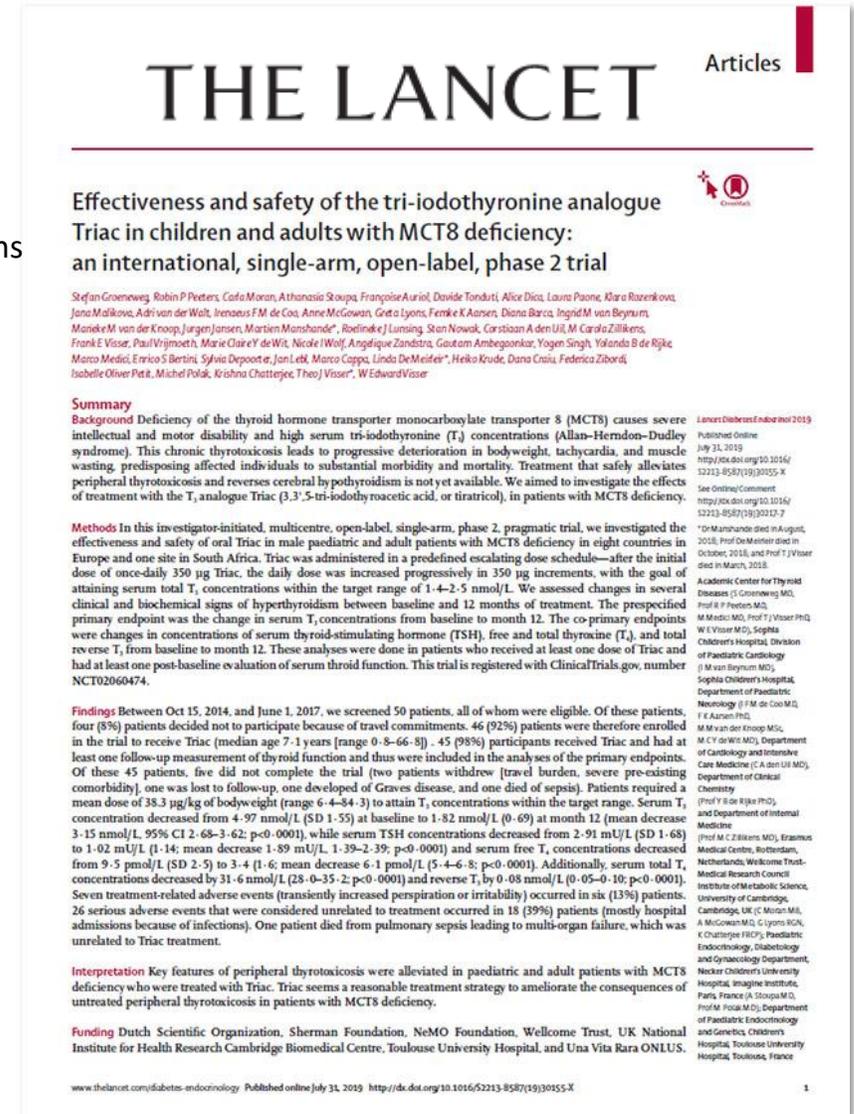
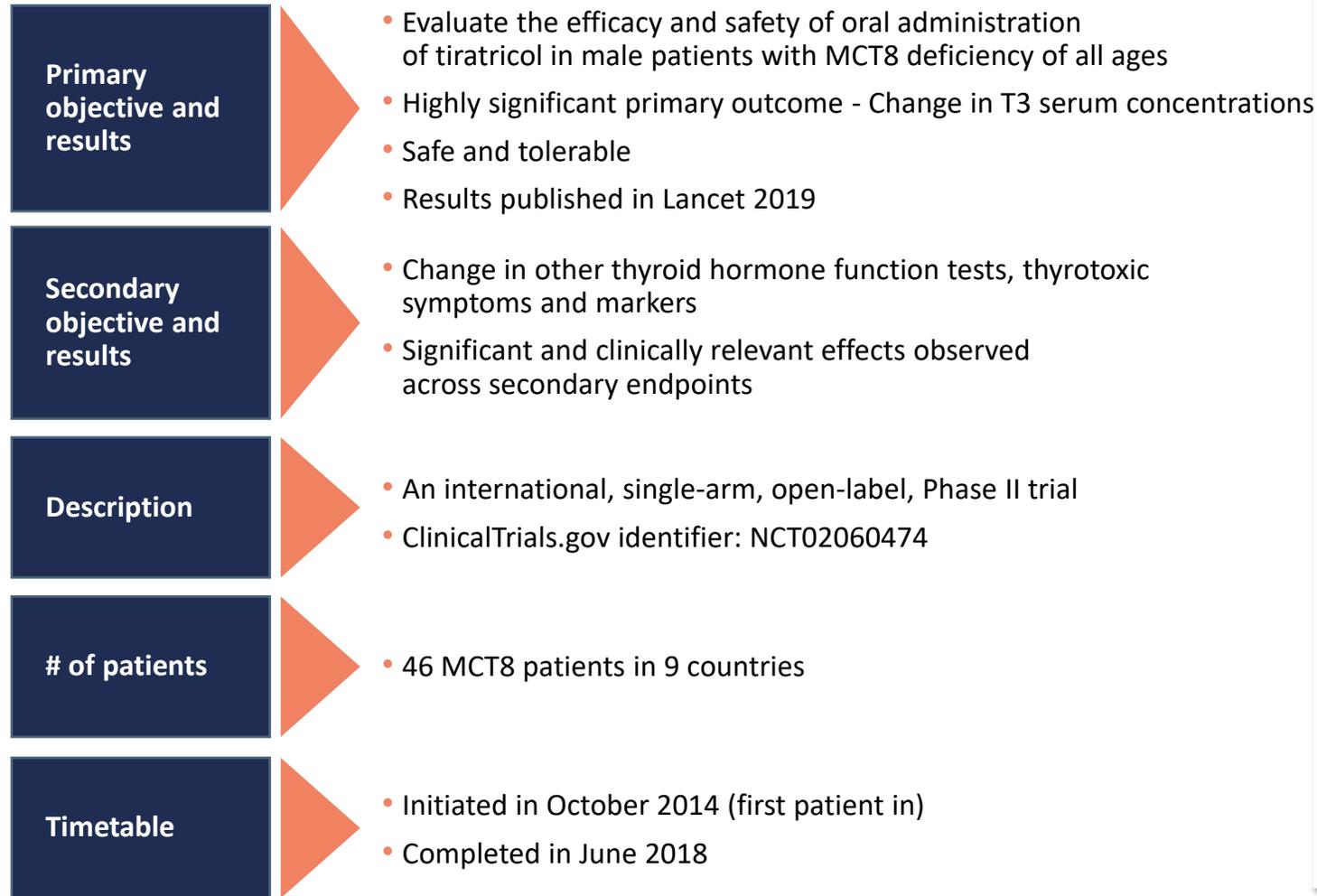


Emcitate (tiratricol) – Addressing the MCT8 deficiency

- Tiratricol is a thyroid hormone analogue with high chemical and structural similarity to T3
- 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 company

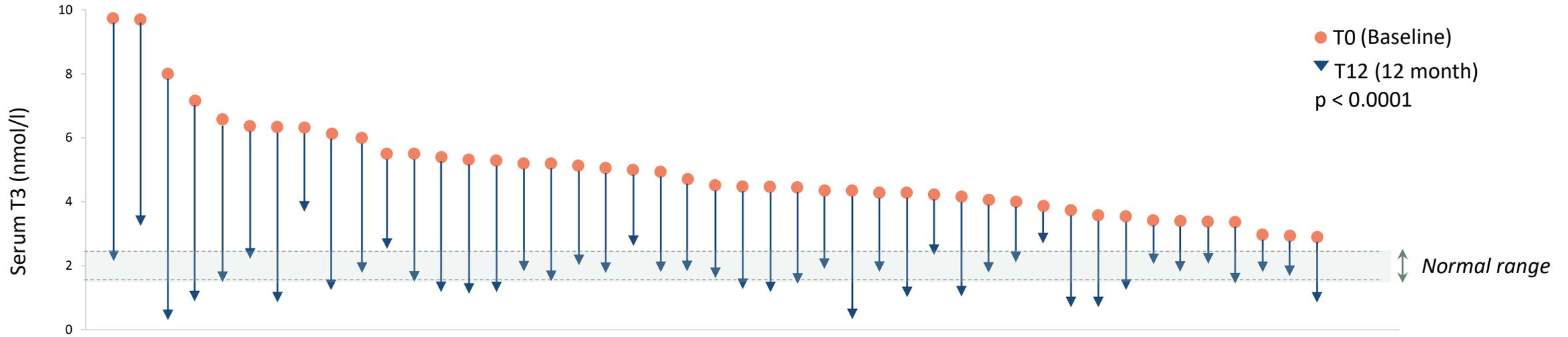


Overview of completed Phase IIb



Consistent, clinically relevant and highly significant results

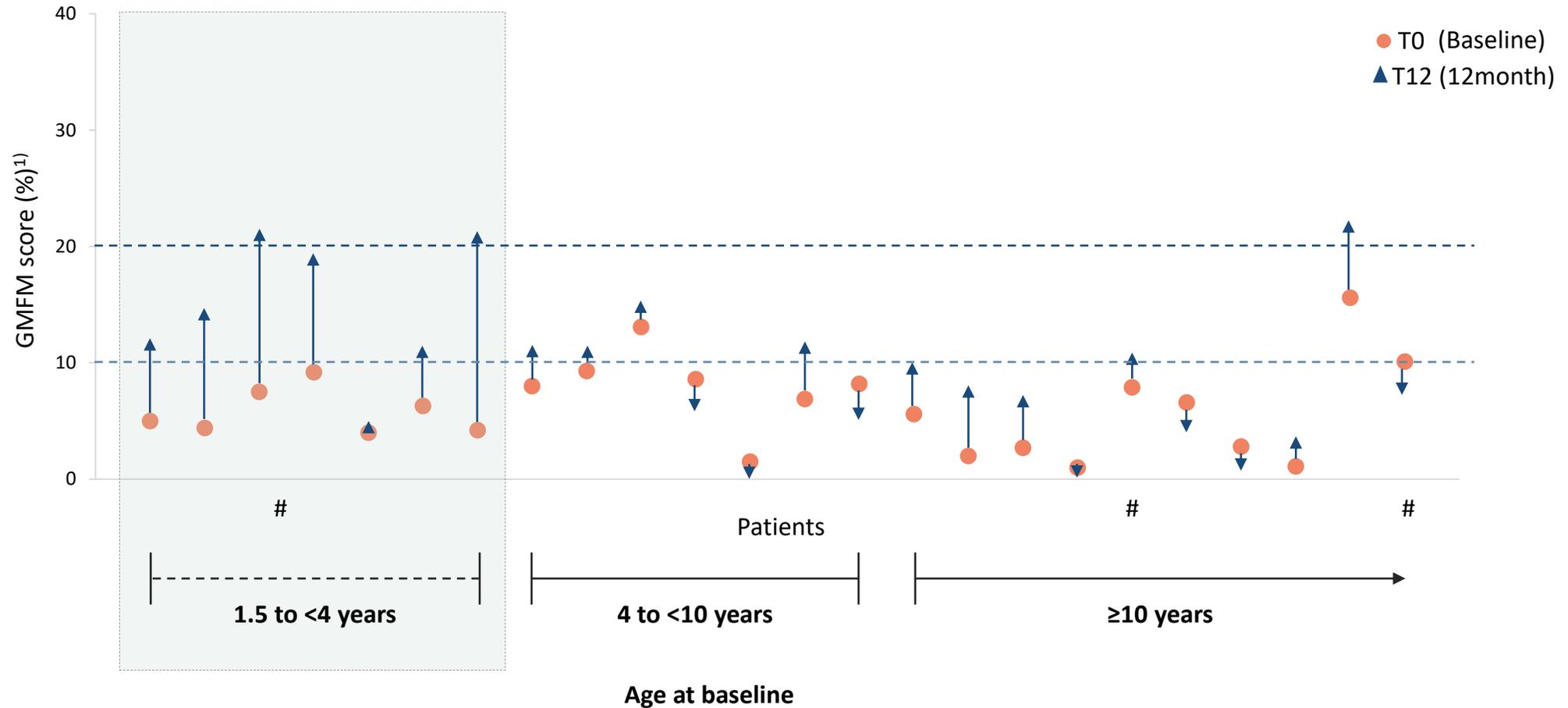
44 out of 48 patients reached target level serum T3 in completed Phase IIb trial



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

Indication of positive effect on neurocognitive development

in the youngest patients which is further studied in ongoing Phase IIb/III trial



Ongoing Phase IIb/III early intervention trial design

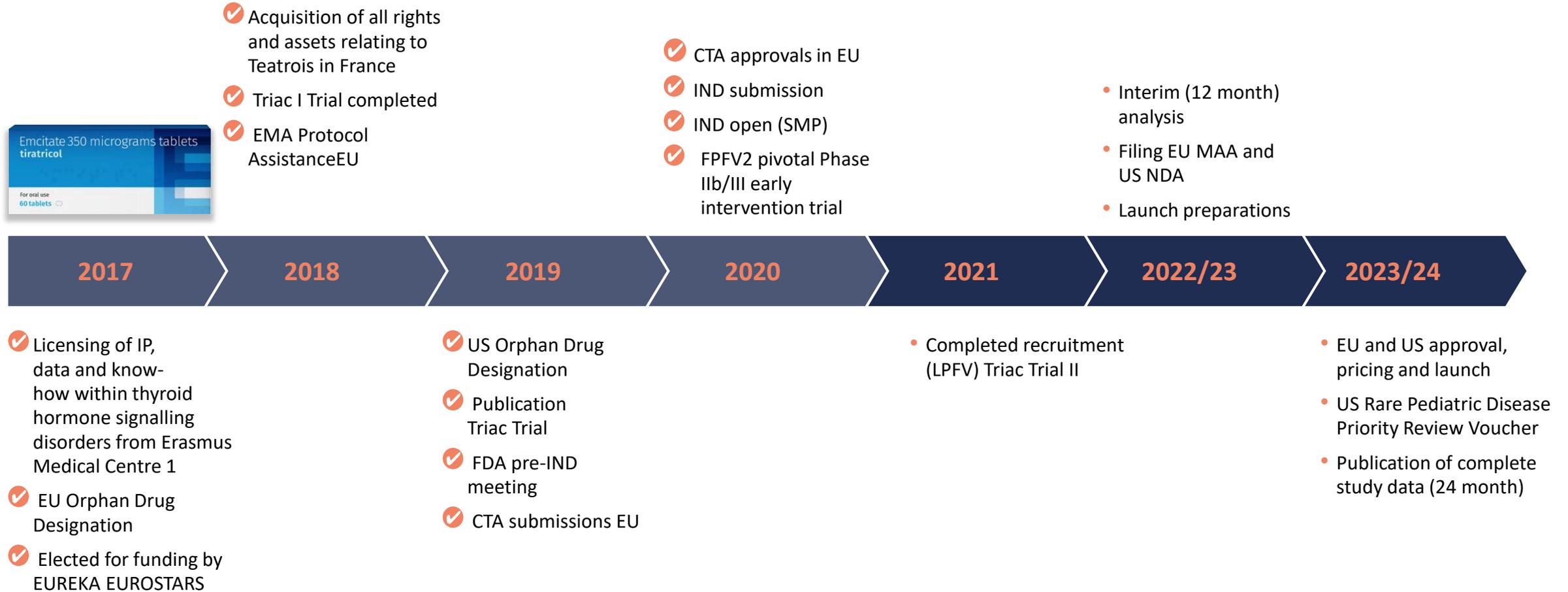
First patient dosed



Primary objective	<ul style="list-style-type: none">• Confirm findings from Triac I Trial in youngest age group• Improvement 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)• Normalisation of thyroid hormone function tests and markers of thyrotoxicosis
Description	<ul style="list-style-type: none">• An open label, multi-centre trial in very young children with MCT8 deficiency• International trial with 10 centres in CZ, DE, IT, UK, FR, NL, US• Design discussed and anchored with EMA and FDA
# of patients	<ul style="list-style-type: none">• 18-22 children 0-30 months of age
Preliminary timetable	<ul style="list-style-type: none">• First Patient First Visit achieved in Dec 2020, LPFV³⁾ planned for Q4 2021• Results from interim analysis at 12 months expected in H2 2022



Emcitate[®] clinical development timeline



Note: (1) Erasmus Medical Centre; (2) First patient first visit; (3) Provided compelling data in 12 month interim analysis of Phase IIb/III early intervention trial

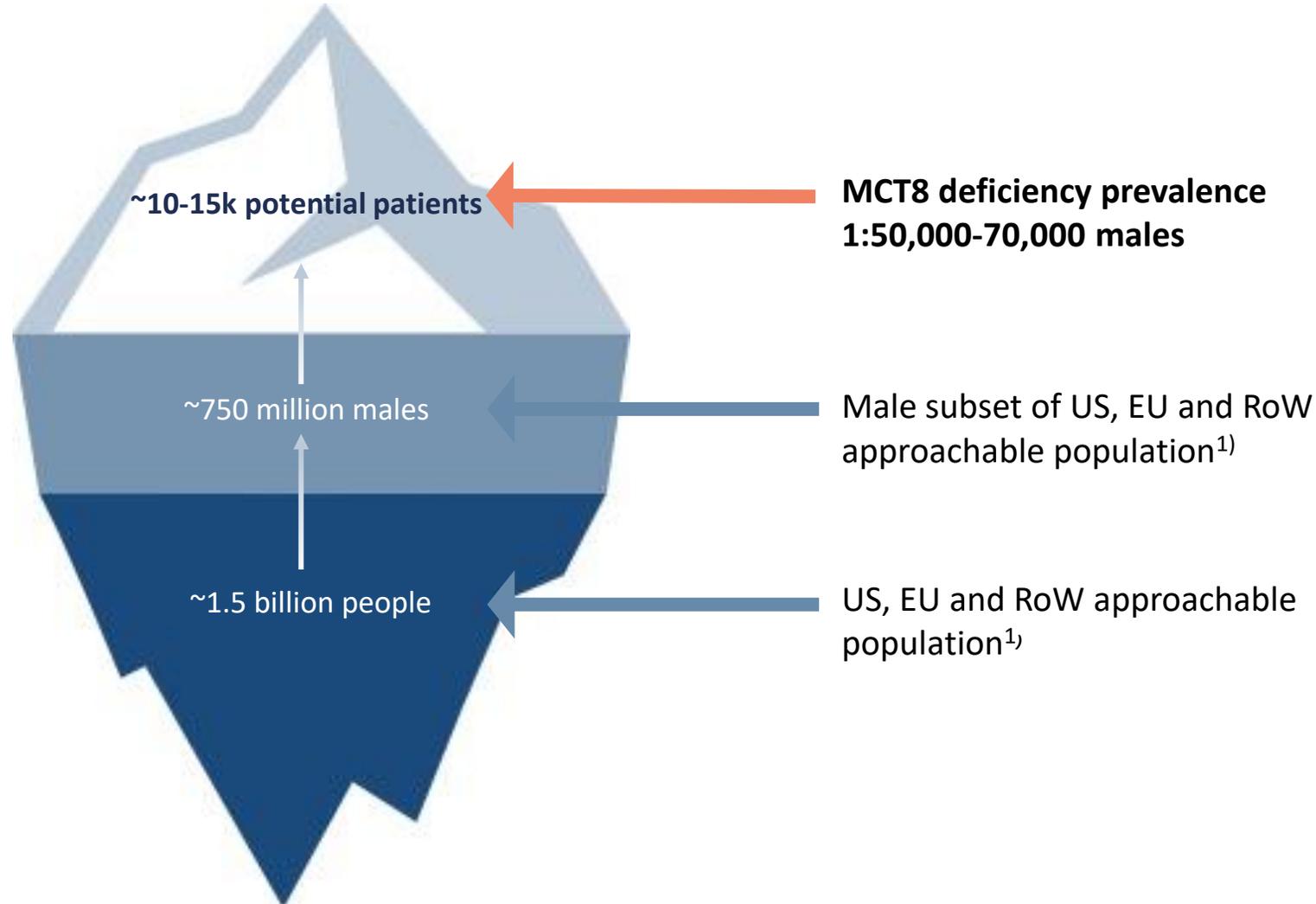


2.

Emcitate[®] - Commercial opportunity

Estimating 10-15k addressable patients globally

No approved treatment for MCT8 deficiency



MCT8 deficiency epidemiology

- At least one new-born diagnosed per 140,000 live births in the Netherlands in past years, corresponding to 1:70,000 males
- Actual number of patients could be higher:
 - Screening study suggests prevalence of 1:50,000 males²⁾
 - Once treatment available, more patients tend to be diagnosed
- **Emcitate supplied on a named patient basis in several countries, in total > 100 patients already**

Emcitate[®] – alleviating patient and societal burden

Aiming to provide value for both patients and society



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

Patients

- Median life-expectancy of MCT8 patients is 35 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



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

Analogue orphan drugs priced at > \$ 250k – 400k per patient

Rapid market penetration with considerable sales already 3rd year in market



- Payers in general accept higher prices for orphan drugs compared to traditional drugs and especially if they;
 - Address an ultra **rare** disease, e.g. prevalence less than 1:50,000 people
 - Target a **severe** disease, i.e. life threatening/debilitating
 - Provide **health gain**, rather than just condition stabilization
- Emcitate fulfills these criteria and can become unique, since no other drugs available or being developed for MCT8 deficiency
- Benchmarks from analogue drugs, comparable to Emcitate in MCT8 deficiency;
 - A global average annual treatment cost of > \$ 250k – 400k
 - Rapid market penetration, considerable sales already 3rd year in market

Emcitate and analogue orphan drugs

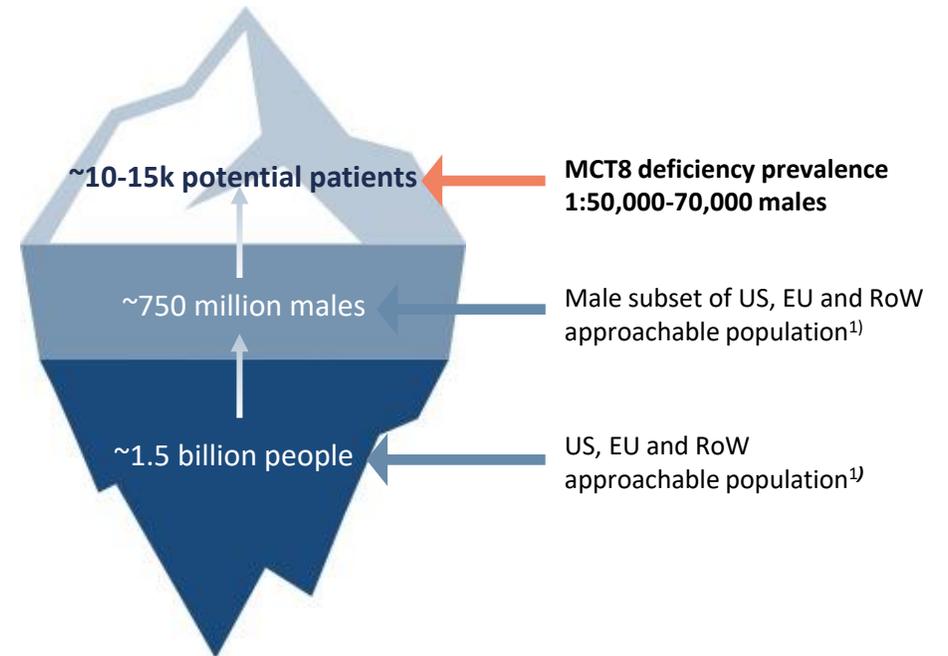
	Vimizim® <i>Recombinant enzyme</i>	Kalydeco® <i>Small molecule</i>	Spinraza® <i>Antisense oligonucleotide</i>	Emcitate (target profile) <i>Small molecule</i>
Disease	MPS IVA	CF with specific mutations	SMA	MCT8 deficiency
Rarity - less than 1:50,000 people	✓	✓	✓	✓
Severity – life threatening/debilitating	✓	✓	✓	✓
Health gain	✓	✓	✓	✓
Global annual treatment cost	> \$400k	> \$250k	> \$350k	TBD
Year of 1st approval	2011	2012	2016	Expected 2023
Global sales 3rd year in market	\$354mn	\$464mn	\$1.7bn	NA
Global sales 2019	\$544mn	\$991mn	\$2.1bn	NA

Emcitate[®] commercial opportunity

– Addressing unmet needs in ultra rare diseases create substantial opportunity



- Significant unmet needs for patients suffering from MCT8 deficiency, i.e. no satisfactory treatments and heavy disease burden
- No other companies developing drugs for MCT8 deficiency
- Analogue benchmarks demonstrate that drugs providing health gains in ultra rare and severe conditions like MCT8 deficiency can achieve
 - global average annual treatment price of > \$ 250k – 400k
 - rapid market penetration with considerable sales already 3rd year in market
 - substantial commercial opportunities



Analogue benchmarks indicate a market potential for Emcitate of > \$1Bn:

- Global average annual treatment cost per patient: > \$250k
- Addressable patients: > 10,000
- Market penetration: 50%

FDA granted Rare Pediatric Disease designation to Emcitate®

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

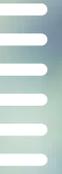


Overview PRV

- The FDA grants Rare Pediatric Disease designation (RPD) to therapies for serious or life-threatening diseases affecting fewer than 200,000 people in the USA.
- PRV program recently prolonged until FY 2026
- Sponsors holding a RPD can apply to receive a US Rare Pediatric Disease Priority Review Voucher (PRV) up on 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.
- By end 2019 22 PRVs for rare pediatric diseases have been awarded by FDA, 12 were sold with individual voucher sale prices ranging from \$67m to \$350m

Examples of PRVs sold

Seller	Buyer	Value	Year
Bavarian Nordics	Undisclosed	\$95M	2019
SOBI	AstraZeneca	\$95M	2019
Bayer Healthcare	argenx	\$100M	2020
Lumos Pharma	Merck	\$100M	2020
Sarepta Therapeutics	Gilead	\$125M	2020



3.

Aladote[®] - clinical development programme

Paracetamol poisoning

– *no adequate treatment for increased-risk patients*



What is paracetamol poisoning?

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

How many does it affect?

- **19 billion** units of paracetamol 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

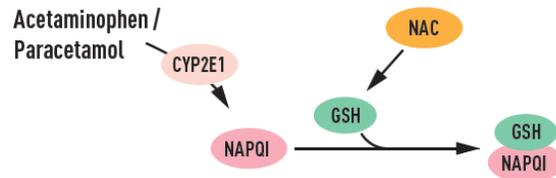
Orphan drug candidate

with clear scientific and mechanistic rationale

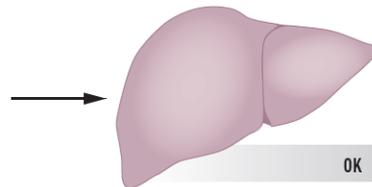


Early presenters (<8h) NAC treatment effective against liver injury

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

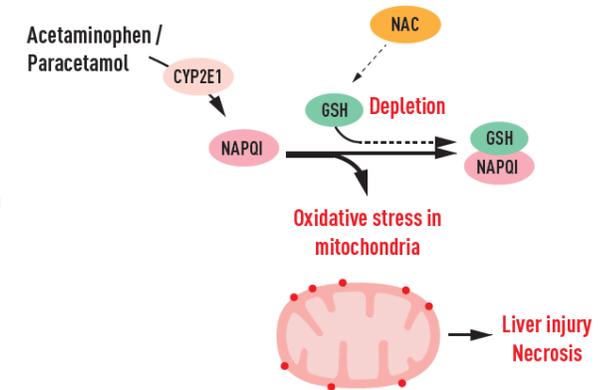


- In most cases NAC effectively prevents liver injury i.e. limited need for Aladote®

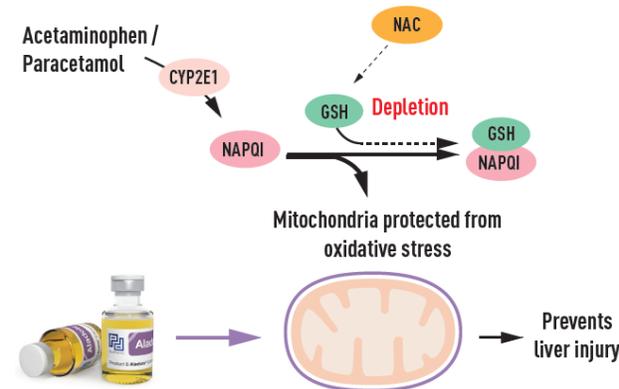


Late presenters (>8h) are at increased-risk for liver injury NAC treatment + Aladote® to prevent liver injury

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

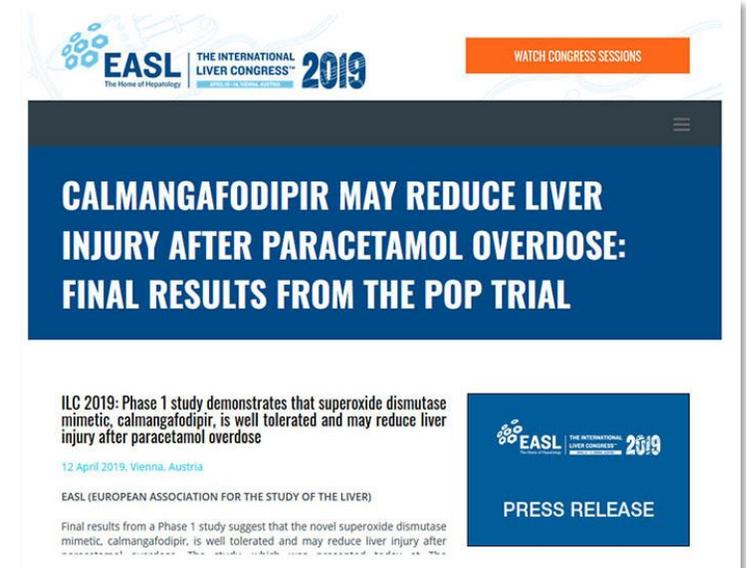


- Aladote®** (calmangafodipir) prevents ROS and RNS formation, restores mitochondrial energy production and **prevents liver injury**



Overview of completed Phase Ib/Ia

<p>Primary objective and results</p>	<ul style="list-style-type: none"> • Met primary endpoint of safety tolerability in the combination of Aladote® and NAC • Results presented at the 58th Annual Meeting of the Society of Toxicology, EASL ILC in April, Vienna and published in Lancet's journal EBioMedicine in 2019
<p>Secondary objectives and results</p>	<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
<p>Description</p>	<ul style="list-style-type: none"> • An open label, rising-dose, randomized study exploring safety and tolerability of Aladote® co-treatment with NAC • ClinicalTrials.gov identifier: NCT03177395
<p># of patients</p>	<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
<p>Timetable</p>	<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

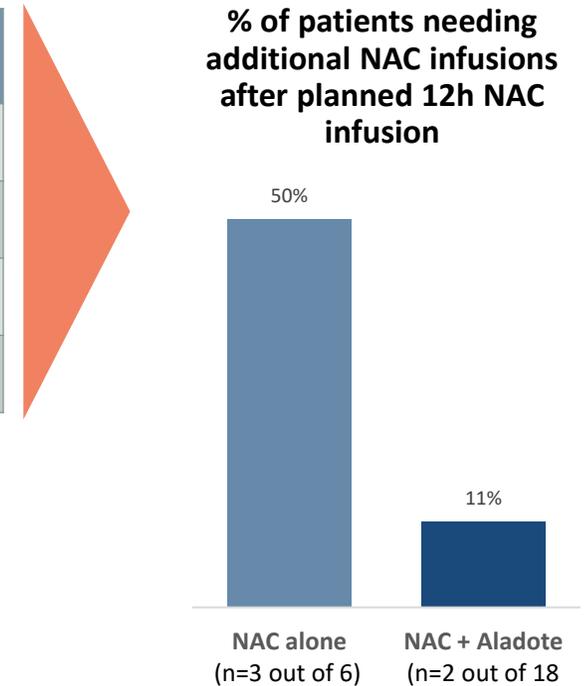
Event	NAC alone	NAC + 2 $\mu\text{mol/kg}$ Aladote	NAC + 5 $\mu\text{mol/kg}$ Aladote	NAC + 10 $\mu\text{mol/kg}$ Aladote
Any AE	6 (100%)	6 (100%)	6 (100%)	6 (100%)
Any SAE	2 (33%)	4 (67%)	2 (33%)	3 (50%)
SAE Starting within 7 days	1 (17%)	1 (17%)	1 (17%)	2 (33%)

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

Liver injury – ALT¹ pre-defined secondary outcome

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

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

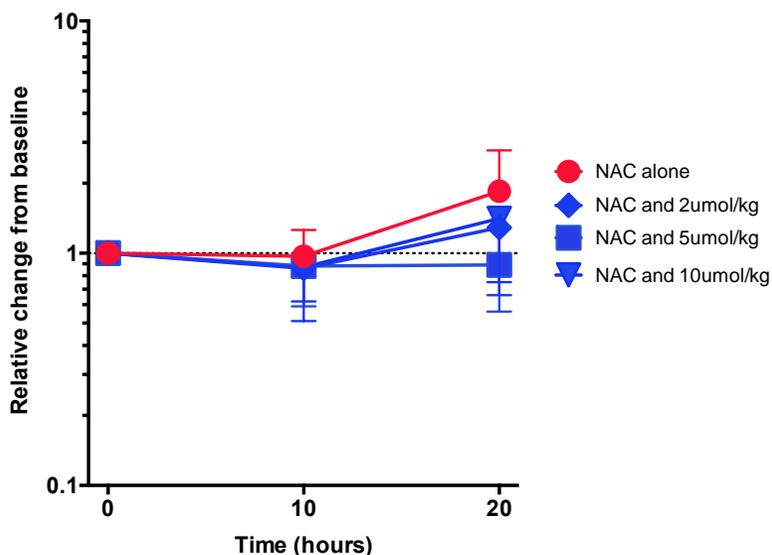


Note: (1) Alanine transaminase (ALT) is a transaminase enzyme also called alanine aminotransferase (ALAT). ALT is found in plasma and in various body tissues especially the liver's hepatocytes. Serum ALT is commonly measured clinically as part of a diagnostic evaluation of hepatocellular injury, to determine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

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

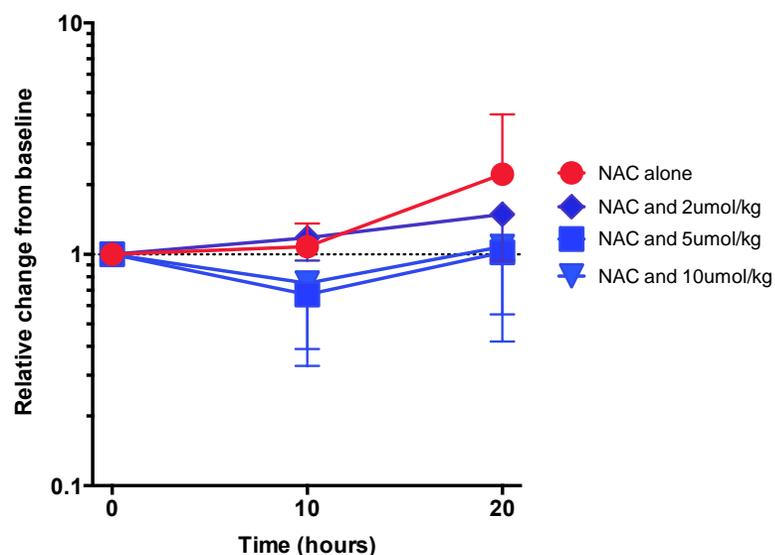


K18



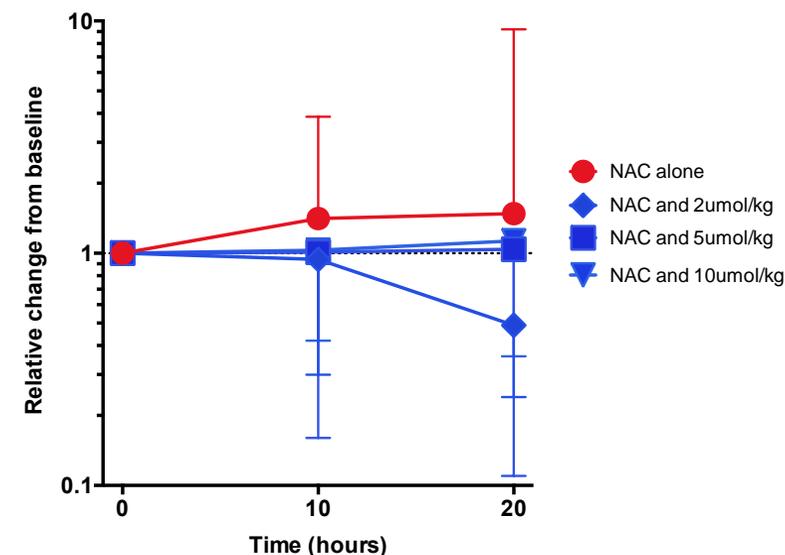
K18 is a measure of cell death and correlate with peak ALT activity during the hospital stay

ccK18



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

miR-22



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

Pivotal Phase IIb/III study for US/EU regulatory submission¹



Efficacy endpoints	<ul style="list-style-type: none">• Primary: Composite of ALT and INR• Number (%) of patients that need further NAC after 21h• Length of hospital stay• Experimental biomarkers, K18, miR-122 and GLDH
Patient population	<ul style="list-style-type: none">• Increased-risk POD patients, Late arrivals (>8h) requiring treatment with 21 hr NAC regime
Description	<ul style="list-style-type: none">• International study in EU, UK and US• IV (bolus) as soon as possible after randomization and after starting NAC (but no later than 4 hours after starting NAC)• 3 arms: Aladote[®] high-dose; Aladote[®] low dose; Placebo
Sample size	<ul style="list-style-type: none">• 225 patients planned
Interim analysis	<ul style="list-style-type: none">• Interim analysis after 50% of patients, that includes a futility analysis and dose selection where the most effective dose will be continued
Preliminary timetable	<ul style="list-style-type: none">• Planned to be initiated H2 2021. COVID situation dependent



Aladote® clinical development timeline



- ✓ US ODD granted
- ✓ Results presented at Society of Toxicology, EASL ILC and Lancet EBiomedicine
- ✓ Regulatory interactions with FDA and EMA

- Interim analysis after 50% of patients included
- Recruitment completed
- Regulatory submissions EU/US

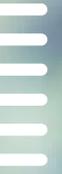


- ✓ Phase Ib/IIa study fully recruited
- ✓ Initial Phase Ib/IIa results
- ✓ Established Scientific Advisory Board
- ✓ Full Phase Ib/IIa results
- ✓ Submission of ODD

- ✓ Design of pivotal study finalised
- ✓ Regulatory interactions FDA, EMA & MHRA
- Initiate pivotal Phase IIb/III study²
- Orphan Drug Designation EU

- First EU/US approval and launch
- Regulatory submissions ROW

Note: (1) Calmangafodipir composition of matter patent expires. (2) COVID 19 situation dependent



3.

Aladote[®] - Commercial opportunity

Estimating at least 175k addressable patients globally



Annual number of POD cases hospitalized and receiving i.v. antidote
(NAC currently the only option)



POD epidemiology

89,000 cases/year of paracetamol overdose in the US and 105,000 cases/year in the UK

- ~50% hospitalized and receive i.v. antidote treatment
- ~25% are late arrivals

Global paracetamol/acetaminophen exposure varies, why POD incidence different between countries

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 in 2010 was 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**

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

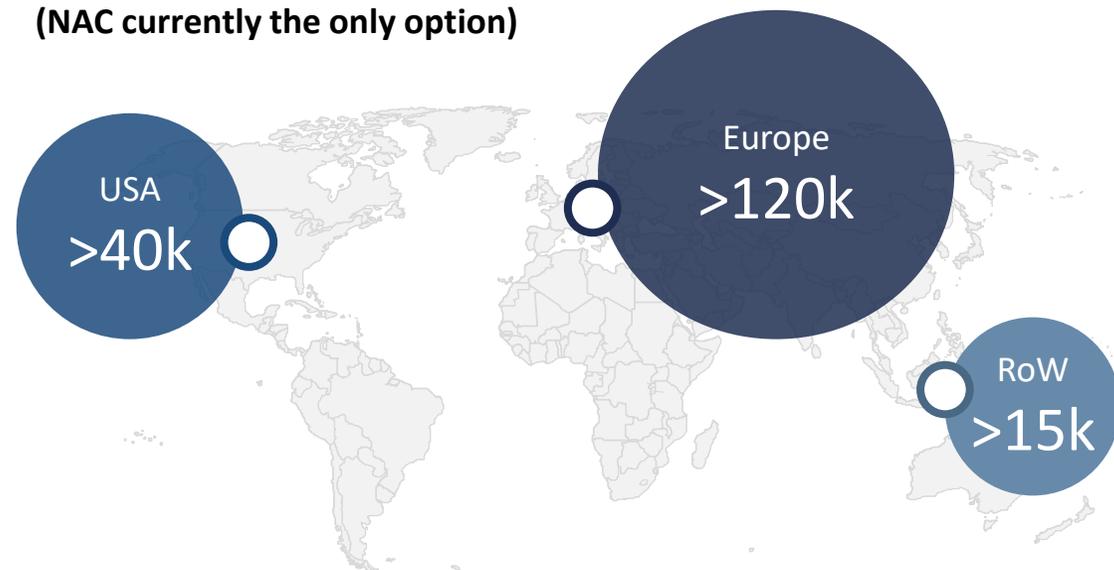
Aladote[®] commercial opportunity

– Addressing unmet needs in antidote market create substantial opportunity



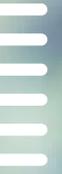
- POD is a life-threatening condition with remaining medical needs
- No effective treatments for high-risk patients, e.g. patients arriving > 8h after ingestion
- No other companies developing drugs for POD
- Opportunity for rapid sales uptake due to national emergency hospital stocking guidelines
- Analogue antidotes priced at \$3.5k – 50k

Annual number of POD cases hospitalized and receiving i.v. antidote (NAC currently the only option)



>\$350mn annual sales opportunity assuming:

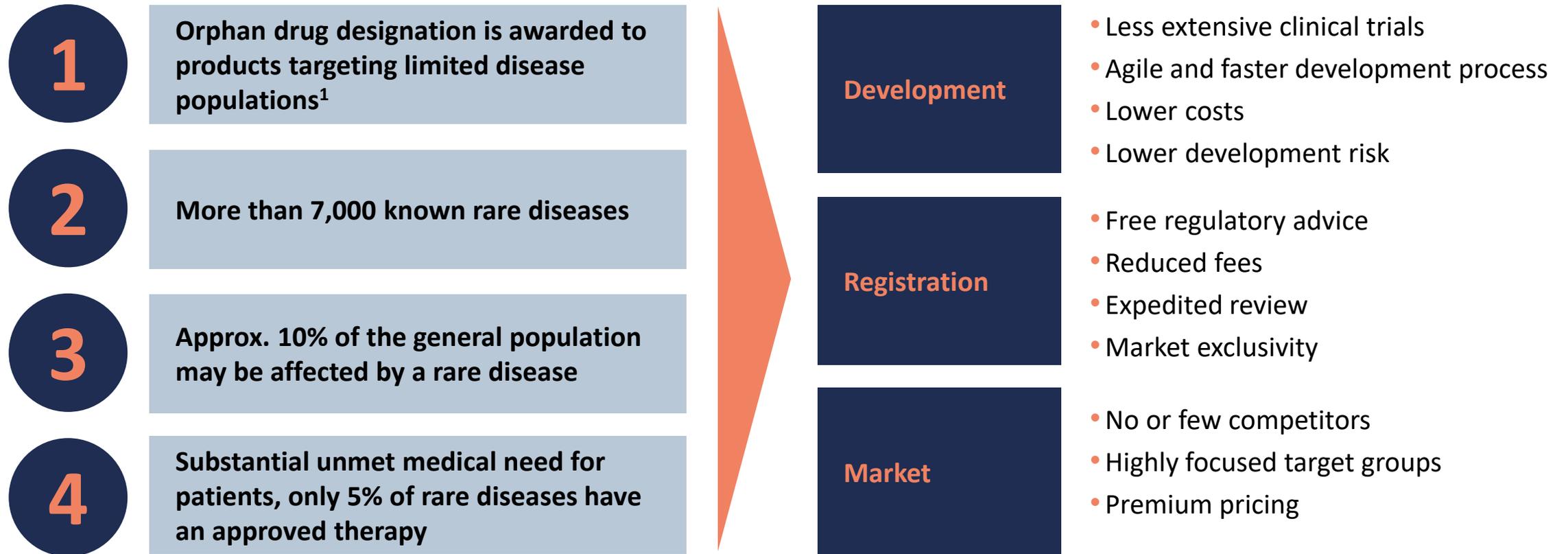
- Global average annual treatment cost per patient: \$5k
- Addressable patients: >175,000
- Market penetration: 40%



4.

The orphan drug segment and path to market

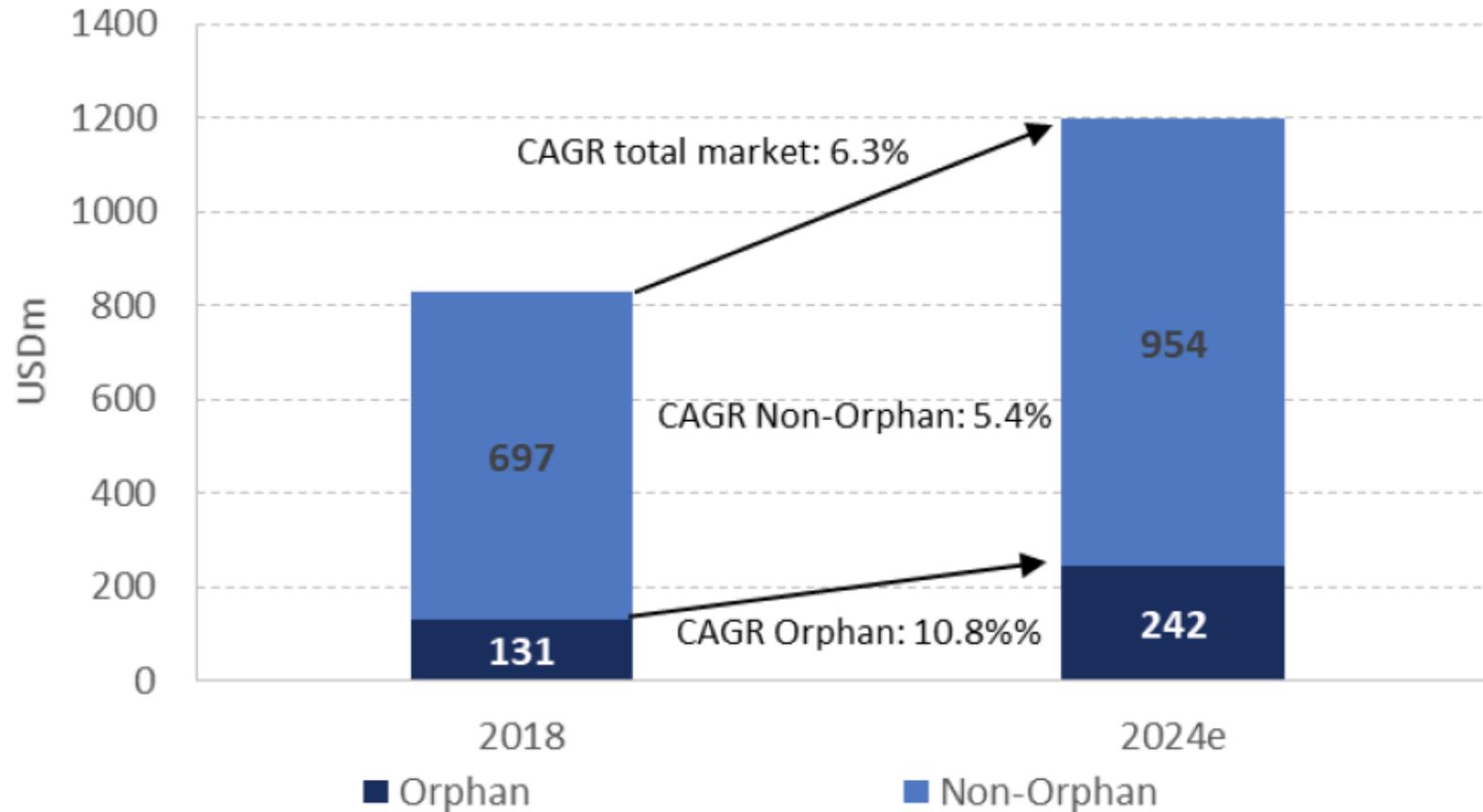
Orphan drug segment – a highly attractive opportunity



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.



Commercialisation of Emciate & Aladote



Strong success factors...

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

...for sustainable, profitable & lean commercialisation

- Plan to build **inhouse commercial capabilities** for launch of Emciate® and Aladote® in EU and US
- **Small and focused footprint** with an estimated < 50 FTEs considered sufficient for both assets
- Retain **larger share of product revenues** over time within company
- **Commercialization** in other territories through **partners**



5.

Summary

Two highly promising orphan drug candidates in one company



Emcitate® – Therapy for genetic disturbance in thyroid hormone signalling with life-long severe disability

- Lead candidate for addressing MCT8 deficiency, a condition with high unmet medical need and no available treatment
- Rare disease which affects 1:70,000 males,
- Obtained Orphan drug designation in the EU and US 2017 and 2019 respectively. **US Rare Paediatric Disease Designation received in November 2020**, eligible for Priority Review Voucher.
- Phase IIb clinical trial completed with significant and clinically relevant effects
- Pivotal Phase IIb/III early intervention trial in young subjects initiated with **first patient dosed in Dec 2020**
- No competing products in clinical development

Aladote® – Prevents acute liver injury caused by paracetamol poisoning

- Paracetamol poisoning is one of the most common overdose with approx. 175,000 hospital admissions in US, EU and RoW approachable population per annum
- No adequate treatment for increased risk patients exists
- Orphan drug designation (ODD) granted in 2019 in the US
- Eligible for ODD in the EU as a results of Brexit, application planned for Q1 2021
- Successful results from Phase Ib/IIa study in paracetamol overdosed patients
- Pivotal Phase IIb/III study planned for marketing authorisation application in both US and EU, ongoing interactions with the regulatory agencies (FDA, EMA and MHRA) to finalize study specific details
- No competing products in clinical development

Late-stage orphan drug pipeline, \$1Bn+ annual sales opportunity



Analogue benchmarks indicate substantial market potential

Emcitate®

Aladote®

Addressable patients¹

> 10,000

Estimate of addressable patients globally with access to western standard health care²

Analogue pricing

> \$250,000

Global average annual treatment cost per patient

Target population

> 175,000

Estimate of addressable patients globally with access to western standard health care²

Pricing assumption

\$5,000

Global average annual treatment cost per patient

Annual sales opportunity:

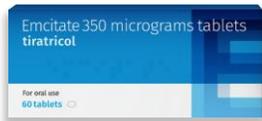
> \$1Bn

If market penetration 50%

Annual sales opportunity:

> \$350mn

If market penetration 40%



COGS assumption: Low single digit percent

Upcoming pipeline milestones



Emcitate®

- ✓ FPFV pivotal Phase IIb/III early intervention trial
- LPFV pivotal Phase IIb/III early intervention trial
- 12m interim analysis Phase IIb/III early intervention trial
- Filing EU/US
- US approval and launch
- US Rare Pediatric Disease Priority Review Voucher
- EU approval and launch



Aladote®

- ✓ Regulatory interactions FDA & EMA
- Initiate pivotal Phase II/III study
- Orphan Drug designation EU
- Interim analysis
- Filing EU/US
- First EU/US approval and launch



New specialised late-stage orphan drug development company



- 1 Dedicated orphan drug development company with two late-stage orphan drug assets: **Aladote[®]** and **Emcitate[®]**
- 2 Highly attractive **orphan drug segment** with potential **>\$1Bn annual sales opportunity**
- 3 Clear path to **market approval in EU and US** within **3 years**
- 4 Plan to **launch** through niche inhouse commercial organization in the EU and US
- 5 Core expertise provides a platform potentially to be leveraged for **additional** late-stage orphan drug projects





A.

Appendix

Leadership team



Nicklas Westerholm

CEO

- Took office in June 2017 and has previously worked in the AstraZeneca Group since 1995 in several global roles in various business areas, most recently as VP Project & Portfolio Management. Prior Nicklas has held positions such as Executive Officer & VP Japan Operations, Director Investor Relations, Head of Global API Supply and Head of Development Manufacture. He has studied Analytical and Organic Chemistry at Stockholm University and Chemical Engineering at KTH, as well as studies at University of Warwick, INSEAD and Harvard Business School.
- Ownership: 27,425 shares and 193,703 warrants



Marie-Louise Alamaa

Interim CFO

- Extensive experience within finance and controlling from public companies. Her previous positions include CFO at Index Invest International AB, various senior finance positions at Crucell Sweden AB (previously SBL Vaccin AB) and Senior Consultant at the listed gaming company Stillfront Group AB. She has studied Economics at the Universities of Uppsala and Stockholm, Sweden, with a particular focus on accounting and auditing



Henrik Krook

VP Commercial Operations

- Appointed VP Commercial Operations in December 2020. He has a broad experience from over 15 years in commercial leadership settings, including both big pharma and biotech. He has previously held senior corporate and commercial advisory roles for biotech companies such as Affibody and senior managerial positions at e.g. Alexion, Novartis and Roche. Henrik has a PhD in immunology from Uppsala University and an Executive MBA from Stockholm School of Economics.
- Ownership: 43,955 (through an insurance solution)



Kristina Sjöblom Nygren*

CMO

- Take office as CMO May 2020 and has previously worked as CMO and Head of Development at Santhera, where she oversaw activities in late-stage clinical development, registration, post-approval commitments and managed access-programs within rare diseases in different therapeutic areas. Previously, Kristina spent 18 years at SOBI, Wyeth and AstraZeneca, where she held a number of senior positions. She has been involved in many different interactions with regulatory bodies such as the US FDA and the EMA including scientific advice and orphan drug applications. Before joining the industry, she worked as a licensed physician in several clinical positions. She holds a Diploma in Pharmaceutical Medicine, and an MD from the Karolinska Institute, Stockholm.*

Jacques Näsström

CSO

- Pharmacist with a Ph.D. in Pharmacology from Uppsala University and with an MBA from the Stockholm School of Economics. He has more than 30 years of experience in the pharmaceutical and biotechnology industry, including a position as Investment Manager at Karolinska Investment Fund and various positions in early drug research at Astra and AstraZeneca. CEO of PledPharma between 2010 and June 2017, before that, Jacques worked as research director at Q-Med AB between 2006-2010
- Ownership: 80,452 shares and 7,748 warrants



Christian Sonesson

VP Product Strategy & Development

- Appointed VP Product Strategy & Development in August 2017 following 13 years at Astra Zeneca. He has broad experience within drug development, including successfully leading products during Phase 3 (FORXIGA® in type 1 diabetes) and of regulatory submissions and defense, bringing new drug candidates to market in different regions (e.g. FORXIGA® in type 2 diabetes, MOVANTIK®, ONGLYZA®-SAVOR, BRILINTA®-PEGASUS and QTERN®). PhD in Biostatistics from Gothenburg University and an Executive MBA from Stockholm School of Economics.
- Ownership: 58,111 warrants

* Joining in May 2021

Board of directors



Håkan Åström

Chairman of the board

- Board member since: 2011
- Other assignments: Chairman of the boards of directors of Affibody Holding AB, Tubulus RP Förvaltning AB and MedCore AB. Board member of Ferrosan Medical Devices A/S and Rhenman & Partner Asset Management
- Ownership: 866 292 shares



Gunilla Osswald

Board member

- Board member since: 2017
- Ph.D. in biopharmacy and pharmacokinetics
- Other assignments: CEO BioArctic AB
- Ownership: -



Sten Nilsson

Board member

- Board member since: 2013
- Professor in oncology with affiliation to the Karolinska Institute (KI), MD, Ph.D.
- Other assignments: Board member of the Swedish Cancer Society Research Council and Rhenman & Partner Asset Management
- Ownership: 1,100 shares



Elisabeth Svanberg

Board member

- Board member since: 2017
- MD, Ph.D., Assoc Professor in surgery
- Other assignments: Chief Development Officer Ixaltis SA. Board member Swedish Orphan Biovitrum (SOBI)
- Ownership: -



Peder Walberg

Board member

- Founder and CEO of Rare Thyroid Therapeutics
- Other assignments: Board Member of Immedica Pharma AB,
- Previous assignments: Founder and CEO, Medical Need, Head of Business Development and Strategy, Swedish Orphan International and SOBI. BoD of Wilson Therapeutics and identified Decuprate for treatment of Wilson disease
- Ownership: 31 858 414 shares

Scientific advisory board



Established for Aladote®



Dr. Richard C. Dart

- Ph.D., Chair of the Department of Medical Social Sciences at Northwestern University Feinberg School of Medicine in Chicago, USA. Expert in evaluations of patient-reported outcomes in clinical trials.



Professor Laura James

- MD, Associate Vice Chancellor for Clinical and Translational Research and Professor of Pediatrics at the University of Arkansas for Medical Sciences (UAMS) and Arkansas Children's Hospital System, USA.



Peter De Paepe

- MD, Professor in clinical pharmacology at the Heymans Institute of Pharmacology at Ghent University, and is currently head of the emergency department of the Ghent University Hospital in Belgium.

Share Register



10 largest shareholders

Name	Capital	Votes	Num. of shares	Verified
Peder Walberg	19.30%	19.30%	31 858 414	2020-12-31
Avla Holding AB	10.04%	10.04%	16 572 442	2020-12-31
Fjärde AP-fonden	8.67%	8.67%	14 311 300	2020-12-31
Peter Lindell	7.39%	7.39%	12 201 219	2020-12-10
Staffan Persson	6.85%	6.85%	11 300 629	2020-12-31
RegulaPharm AB	5.97%	5.97%	9 846 730	2020-12-31
Nyenburgh Holding B.V.	2.26%	2.26%	3 735 583	2020-12-10
Avanza Pension	2.16%	2.16%	3 571 010	2020-12-31
Thomas Elderred	1.36%	1.36%	2 251 674	2020-12-31
Mats Blom	1.20%	1.20%	1 988 227	2020-12-31
Total 10	65.21%	65.21%	107 637 228	
Total number of owners	5,476			2020-12-31
Total number of shares	165,068,560			2020-12-31



Thank you!

Egetis Therapeutics
egetis.com