EGETIS THERAPEUTICS

WE CARE

FOR THE RARE

Egetis Investor Day 2023

December 19, 2023

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

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Agenda: Egetis Investor Day 2023

Time (CET/ET)	Subject	Presenter(s)
15:00/9.00am	Welcome, corporate strategy and overview	Nicklas Westerholm, CEO
15:15/9.15am	Development of *Emcitate* for MCT8 deficiency patients	Westerholm
15:30/9.30am	MCT8 deficiency and the unmet medical need	Dr. Andrew Bauer, CHOP, Philadelphia, PA
15:50/9.50am	Q&A	Bauer & Westerholm
16:00/10.00am	Global plans for commercializing *Emcitate*	Henrik Krook, VP Commercial
16:15/10.15am	Understanding MCT8 deficiency patients' & caregivers' needs	Nigel Nicholls, Global Patient Advocacy Director
16:30/10.30am	Improving disease awareness of MCT8 deficiency	Peter Verwaijen, Global Head of Marketing & Brand Strategy
16:45/10.45am	US launch preparations for *Emcitate*	Anny Bedard, President Egetis North America
17:00/11.00am	Q&A	Krook, Nicholls, Verwaijen, Bedard, Westerholm
17:10/11.10am	Break	
17:30/11.30am	RTH-beta and the unmet medical need	Dr. Carla Moran, University College Dublin
17:50/11:50am	Q&A	Moran & Westerholm
18:00/12:00pm	Concluding remarks	Westerholm

Presenting remotely

Building a sustainable orphan drug company

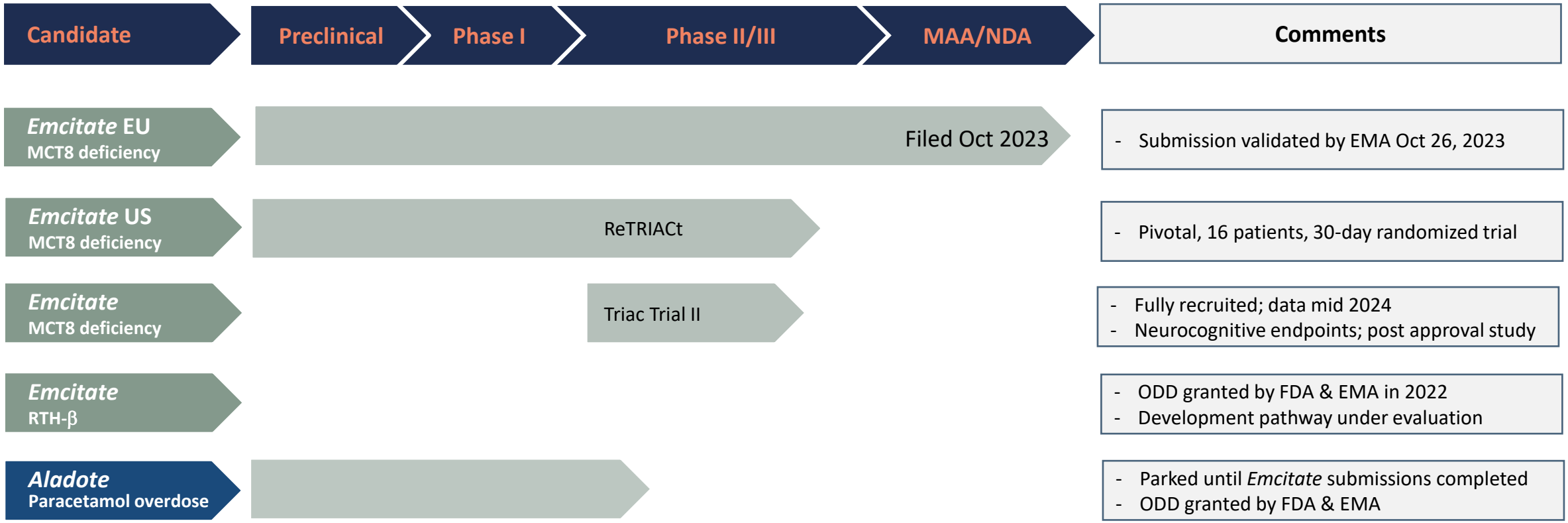
- Successfully develop *Emcitate* for EU & US approvals in 2024/25 and *Aladote* post 2026
- Commercialize *Emcitate* and *Aladote* through an inhouse organization in Europe/ North America and partnerships in RoW
- Realize the full potential of our products via life-cycle management
- Ensure fast and 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

Pipeline overview

Emcitate MAA filed in Oct 2023



Leadership team with global experience & proven track record

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

Yilmaz Mahshid, PhD
CFO
- Joined 2021
- Investment Manager & Controller at Industrifonden
- Sell-side analyst at Pareto & Öhman
- CEO Medivir

Christian Sonesson, PhD
VP Product Strategy & Development
- Joined 2017
- AstraZeneca 13 years
- Late-stage development expertise from FORXIGA, MOVANTIK, ONGLYZA, BRILINTA & QTERN

Desiree Luthman
VP Regulatory Affairs
- Joined 2023
- Global regulatory professional, >25y experience
- Passage Bio, Verona Pharma, Sanofi, BMS, Celgene, AstraZeneca

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

Henrik Krook, PhD
VP Commercial Operations
- Joined 2020
- Commercial roles at Alexion, Novartis, Roche and Affibody

Laetitia Szaller
General Counsel & Head of Compliance
- Joined 2023
- Senior legal roles at AM Pharma, UCB & Zoetis

Katayoun Welin-Berger, PhD
VP Technical Operations
- Joined 2023
- VP Operations at Calliditas Therapeutics
- Previously at BioGaia and AstraZeneca

Anny Bedard
President Egetis North America
- Joined 2022
- Commercial leadership roles at Shire and Sarepta

Nils Hallen
Global Head of HR
- Joined 2021
- Adjunct professor in work & organizational psychology

Karl Hård, PhD
VP IR & Business Development
- Joined 2022
- Redx Pharma, Optimum Strategic Communications, Kiadis, AstraZeneca

Board of directors

Thomas Lönngren

Chair of the board since 2021

- Shares in Egetis: 283,158
- MSc in social and regulatory pharmacy and a degree in Pharmacy, University of Uppsala.
- Previously Executive Director of the European Medicines Agency
- Other assignments: Board member Compass Pathways & NDA Group

Peder Walberg

Board member since 2020

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

Gunilla Osswald

Board member since 2017

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

Mats Blom

Board member since 2021

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

Elisabeth Svanberg

Board member since 2017

- Shares in Egetis: 37,676
- MD, PhD, Assoc Professor in surgery
- Other assignments: Chief Development Officer Ixaltis SA. Board member Leo Pharma, Amolyt Pharma, Galapagos and EPICS Therapeutics

Behshad Sheldon

Board member since 2023

- Shares in Egetis: 0
- BS in neuroscience
- Other assignments: Chair of the Board of FORCE (Female Opioid Research and Clinical Experts) in Princeton, NJ, Board Member, Camurus AB and Maxona Pharmaceuticals; EVP & MD, Biotech Value Advisors

Egetis has recently delivered transformative milestones

Jul 24

First patient recruited in ReTRIACt study, which is pivotal for US NDA

Oct 9

Submitted application for regulatory approval of *Emcitate* for MCT8 deficiency in EU (MAA)

Oct 10

Secured long-term financing of SEK 462m, adding top-tier US specialist investor as largest shareholder

- SEK 172m equity private placement (SEK 155m subscribed by Frazier Life Sciences)
- SEK 290m debt financing (BlackRock; EUR 10m & EUR 15m tranches)

Nov 10

License agreement with Fujimoto for *Emcitate* in Japan

Nov 30

EUR 10m draw down of debt facility

Submission of application for regulatory approval in EU (MAA) for *Emcitate* for treatment of MCT8 deficiency

- **Marketing Authorisation Application (MAA) submitted and validated by the EMA in October**
- Application validated and found to be complete by the EMA on October 27, starting the clock for formal regulatory review
- The median review time for MAAs in the EU is around 13-14 months
- Orphan Drug Designation will provide 10 years' of market exclusivity within the EU following approval of MAA

Egetis submits marketing authorisation application for Emcitate for treatment of MCT8 deficiency to the European Medicines Agency

October 9, 2023

- Submission based on efficacy data from two studies in a total of 86 MCT8 deficiency patients with up to 6 years of treatment with Emcitate
- To date an accumulated 500 patient years of Emcitate treatment have been gathered in patients with MCT8 deficiency
- If approved, Emcitate would become the first treatment for MCT8 deficiency

Stockholm, Sweden, October 9, 2023. Egetis Therapeutics AB (publ) ("**Egetis**" or the "**Company**") (Nasdaq Stockholm: EGTX), today announced that the Company has submitted a marketing authorisation application (MAA) to the European Medicines Agency (EMA) for Emcitate (tiratricol) for the treatment of MCT8 deficiency. This is an important step towards bringing the first approved treatment for MCT8 deficiency to patients and a transformative milestone for the Company.

Egetis announces EMA validation of Marketing Authorisation Application for Emcitate for the treatment of MCT8 deficiency

October 27, 2023

Stockholm, Sweden, October 27, 2023. Egetis Therapeutics AB (publ) ("**Egetis**" or the "**Company**") (Nasdaq Stockholm: EGTX), today announced that its Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for *Emcitate* (tiratricol) for the treatment of MCT8 deficiency has been validated. On October 9, 2023, Egetis announced the submission of the MAA. EMA performs a validation of the applications it receives. The objective is to make sure all essential regulatory elements required for scientific assessment are included in the application prior to the start of the review procedure. The *Emcitate* MAA is as of October 26, 2023, under review by the Committee for Medicinal Products for Human Use (CHMP).

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

Announcement published on Oct 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 10 million (“Tranche A”) and EUR 15 million (“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

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: EGTX), 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.

Development of Emcitate® for MCT8 deficiency patients

Emcitate regulatory pathway to submissions in EU and US

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

Included in MAA in EU submitted on October 9, 2023

Included in NDA in US with the Fast Track Designation

Not required for MAA or NDA submissions

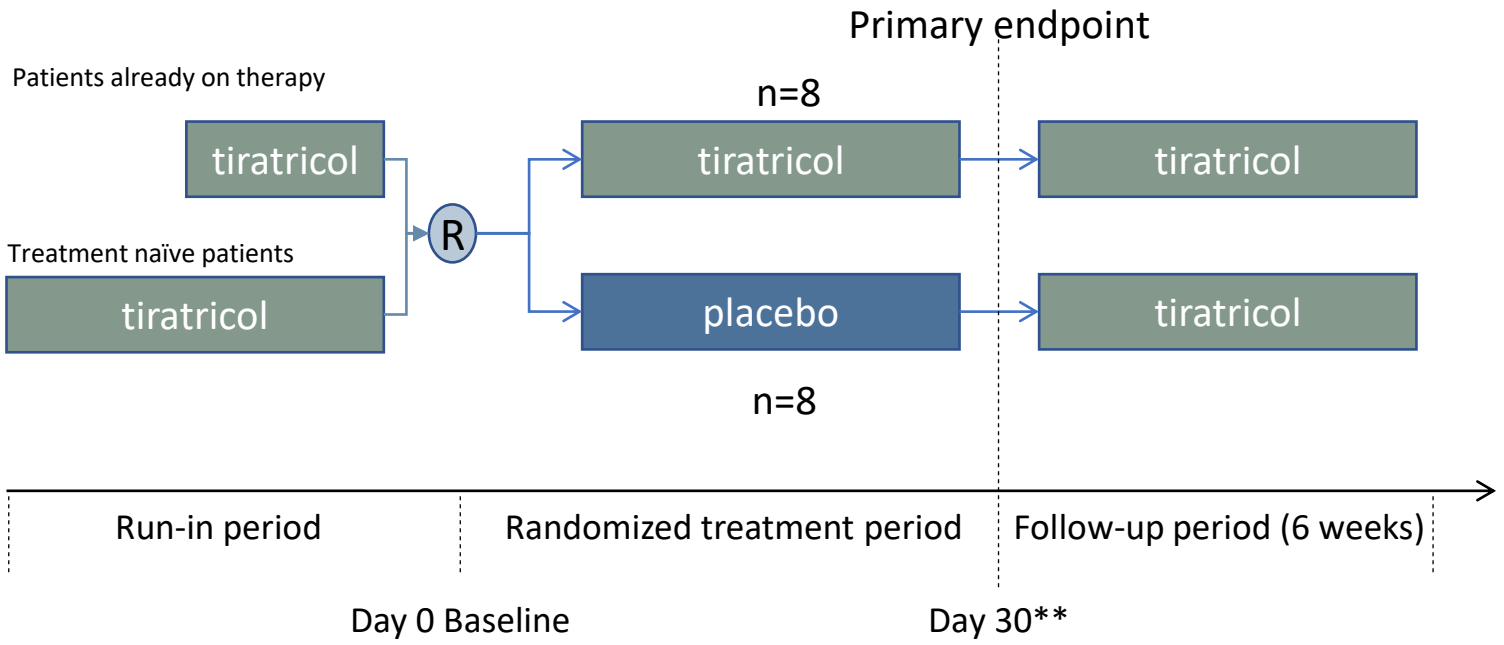
Triac Trial I	EMC cohort study	Natural history	ReTRIACt Trial	Triac Trial II
• Completed 2018 (Groeneweg, 2019) • Open-label, international, multi-centre study • N= 46	• Completed 2021 (van Geest, 2022) • N= 27 from Triac Trial I & N= 40 new pts from managed access program	• Retrospective data, 2003 to 2019 (Groeneweg, 2020) • N= 151	• N= 16 • Placebo controlled	• Fully recruited 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

Design of the ReTRIACt clinical trial

Requested by the FDA as pivotal for the New Drug Application in the USA

- A 30-day, randomized placebo-controlled withdrawal study in 16 patients
- Design agreed with FDA
- The study allows for inclusion of both 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

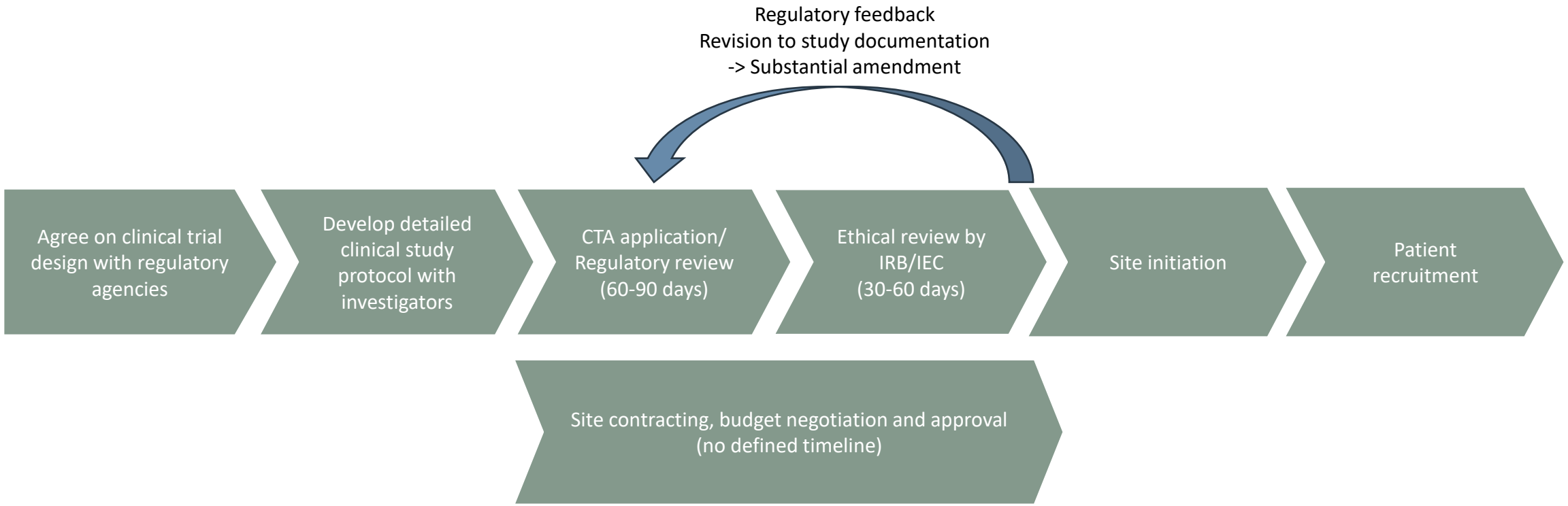


Primary endpoint: Proportion of participants who meet the rescue criterion (T3>ULN*) during the 30-day double-blind randomized treatment period

* ULN: Upper Limit of Normal

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

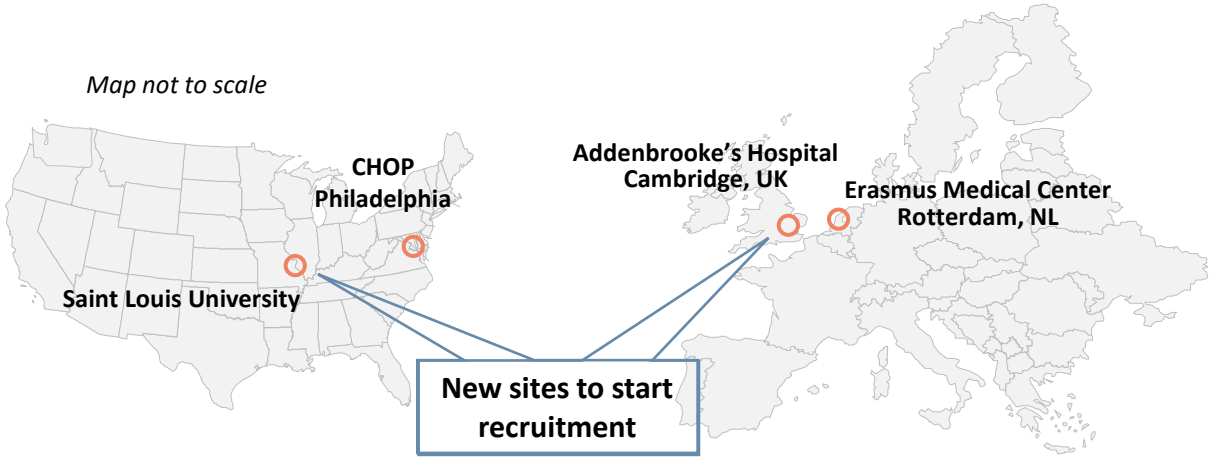
Roadmap to site initiations in the ReTRIACt trial

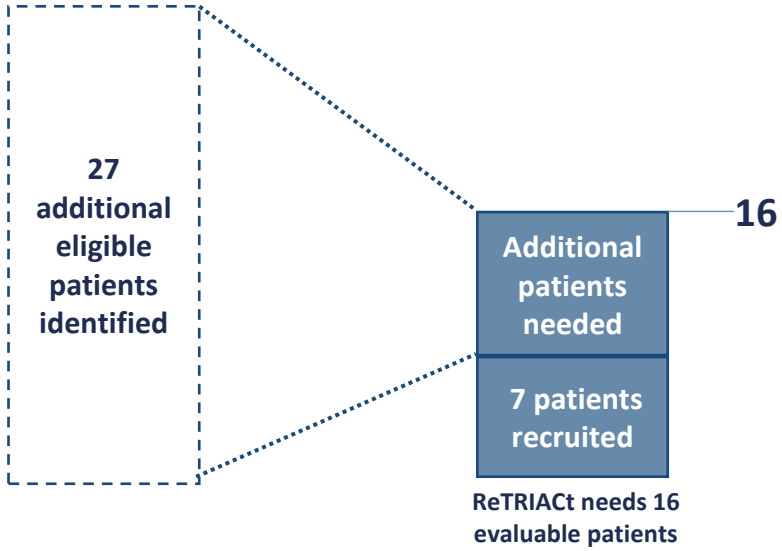


- Longer than anticipated review times for IRB/IEC and site contracting as well as the need for a substantial amendment of study documentation led to significant delays in site initiation.

CTA = Clinical Trial Application, IRB = Institutional Review Board, IEC = Independent Ethics Committee

Current status of ReTRIACt trial





- 7 patients recruited to date of 16 evaluable patients needed
- 2 sites (EMC & CHOP) have been recruiting during autumn
- 4 sites will be recruiting going forward
- 27 Eligible patients identified and remaining for recruitment going forward consists of 8 on-treatment and 19 treatment naïve patients
- Several factors affect completion of the ReTRIACt study e.g. patient type and availability, recruitment capacity, titration time

⇒ **Egetis has taken steps to mitigate these factors and will update the market as soon as recruitment has been completed, and subsequently when top-line results and NDA filing can be expected**

Regulatory features of *Emcitate* for MCT8 deficiency

ODD

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

Fast track

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

PRV

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

MAA
NDA

MAA: Submitted in October ’23
NDA: Small confirmatory study agreed with FDA

ODD

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

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

FDA granted Rare Pediatric Disease designation to Emcitate®

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

Overview of PRV

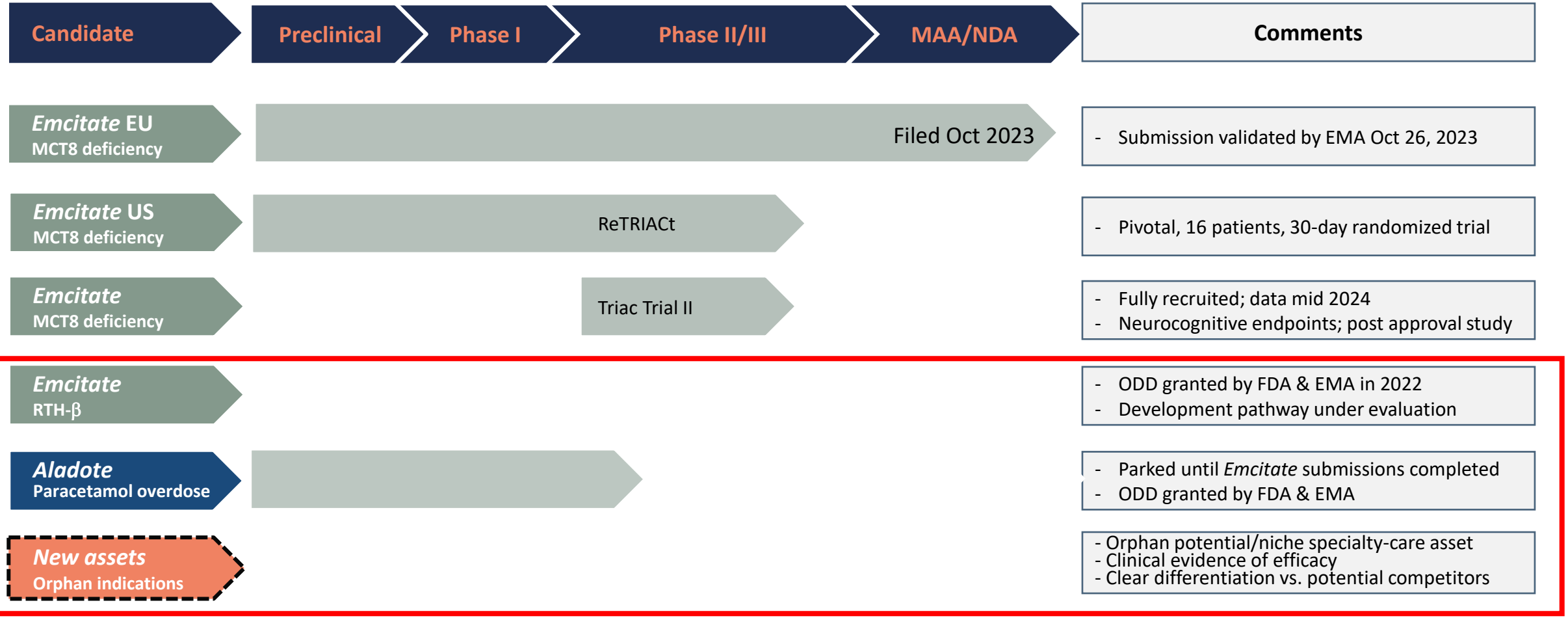
- The FDA grants Rare Pediatric Disease designation (RPD) to therapies for serious or life-threatening diseases affecting fewer than 200,000 people in the USA.
- Sponsors holding a RPD can apply to receive Priority Review Voucher (PRV) upon approval.
- PRV program prolonged until 2026.
- Provides accelerated FDA review of a new drug application for another drug candidate, in any indication, shortening time to market in the US.
- The voucher may be sold or transferred to another sponsor.
- During 2021-23 PRVs have been sold ranging from $100m-$110m.

Examples of PRVs sold

Seller	Buyer	Value	Year
Liminal Biosciences	Undisclosed	$105M	2021
Mirum Pharmaceuticals	Undisclosed	$110M	2021
Rhythm Pharmaceuticals	Undisclosed	$100M	2021
Albireo	Undisclosed	$105M	2021
Biomarin	Undisclosed	$110M	2022
BridgeBio	Undisclosed	$110M	2022
Mallinckrodt	Novartis	$100M	2022
Marinus Pharmaceuticals	Novo Nordisk	$110M	2022
Sarepta	Undisclosed	$103M	2023

Under the terms of the acquisition of RTT in Q4 2020, 50% of the potential net proceeds from a sale of a PRV related to Emcitate should be paid as an earnout to the sellers of RTT

Building a sustainable rare disease company



Agenda: Egetis Capital Markets Day 2023

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16:15/10.15am	Understanding MCT8 deficiency patients' & caregivers' needs	Nigel Nicholls, Global Patient Advocacy Director
16:30/10.30am	Improving disease awareness of MCT8 deficiency	Peter Verwaijen, Global Head of Marketing & Brand Strategy
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18:00/12:00pm	Concluding remarks	Westerholm

MCT8 Deficiency Efforts to Address Unmet Needs for Patients and Care-Givers

Andrew J Bauer, MD

December 2023

What is MCT8 deficiency?

Historical milestones in MCT8 deficiency:

1944
Allan-Herndon-Dudley syndrome (MCT8 deficiency) first described in a family of 24 males spanning 6 generations[1]

1990
Affected gene localized to X chromosome.[1,2] Clinical symptoms described in detail[1,2]

2003
Identification of MCT8 as a specific thyroid hormone transporter[3]

2004
Association of disorder to mutation in the *SLC16A2* gene established[4,5]

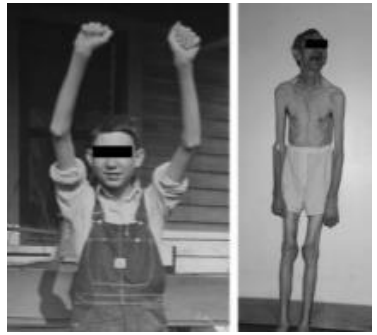


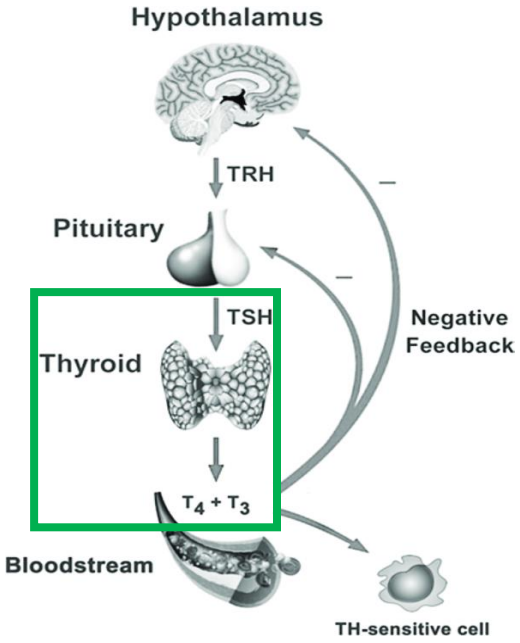
Image from Schwartz et al. (1990)[2]

MCT8, Monocarboxylate transporter 8.

1. Stevenson RE, et al. Am J Hum Genet. 1990;47(3):446–53; 2. Schwartz CE, et al. Am J Hum Genet. 1990;47(3):454-8; 3. Friesema EC. J Biol Chem. 2003;278(41):40128–35; 4. Dumitrescu AM. Am J Hum Genet. 2004;74(1):168–175; 5. Friesema EC, et al. Lancet. 2004;364(9443):1435–7.

Thyroid hormone is key to regulating many processes in the body

- **Thyroid hormone** (TH) is essential for the formation and functioning of nearly all tissues[1–4]
  - Key role in the regulation of metabolism and tissue regeneration



BRAIN
Development and function
Myelination

PITUITARY
TSH production
Growth hormone

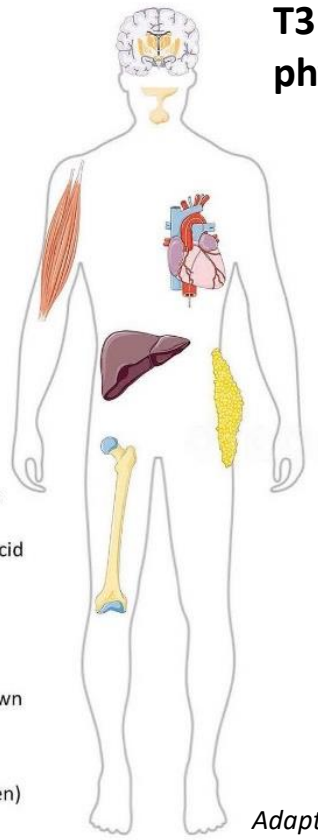
HEART
Cardiac output
Heart rate
Hypertrophy

MUSCLE
Protein catabolism
Energy expenditure

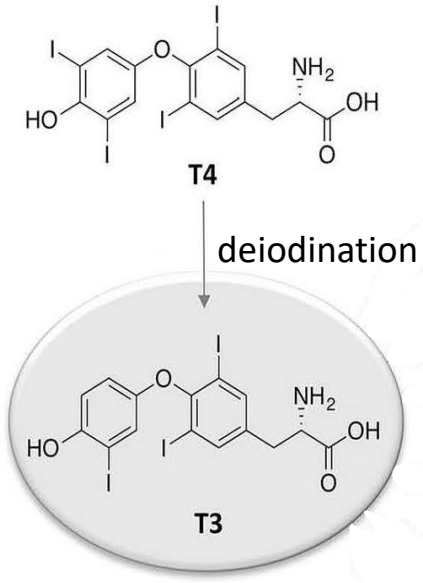
LIVER
Mitosis
Gluconeogenesis
Fatty acid β-oxidation/synthesis
Cholesterol synthesis/uptake
Cholesterol conversion to bile acid
Apolipoprotein A1
HDL receptor (SR-B1)

ADIPOSE TISSUE
Lipolysis
Adaptive thermogenesis in brown adipose tissues

SKELETAL SYSTEM
Growth and maturation (children)
Bone resorption (adult)



T3 is critical in multiple physiologic processes[4]



Adapted from Saponaro et al. (2020)[4]

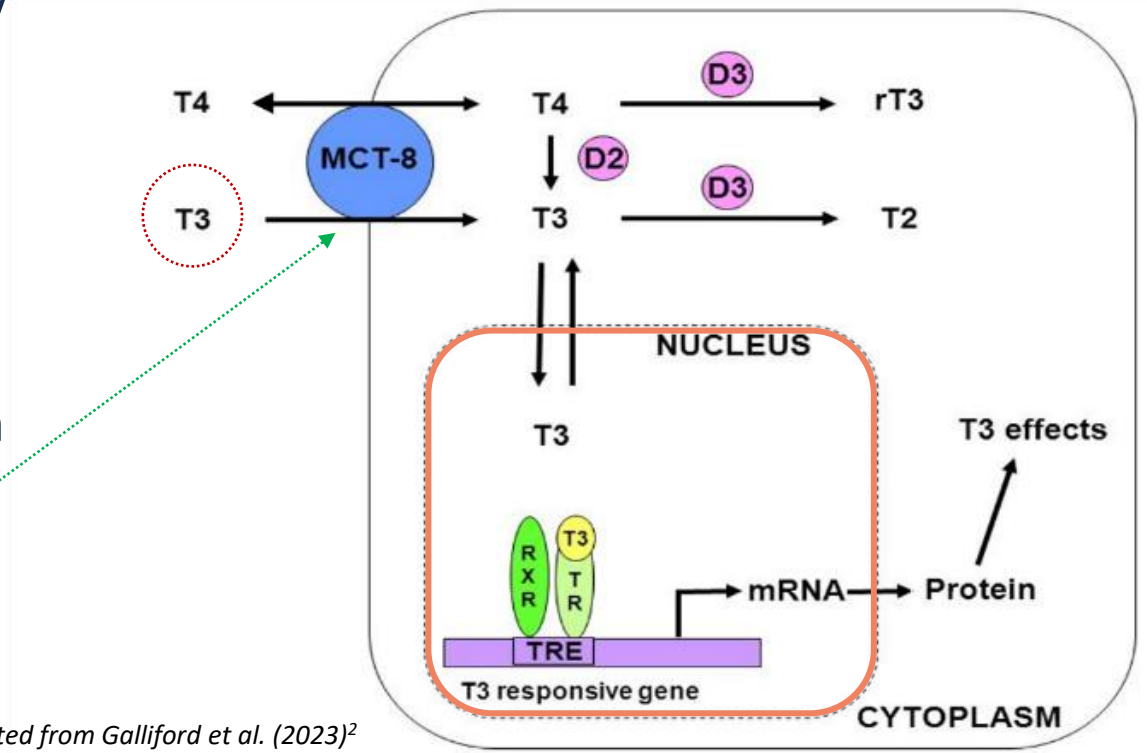
HDL; high-density lipoprotein; T3, triiodothyronine; T4, thyroxine; TH, thyroid hormone; TSH: thyroid-stimulating hormone.

1. Groeneweg S et al. Endocr. Rev 2020;41(2): 146–201; 2. van Geest FS et al. Endocrine. 2021;71:689–695; 3. van Geest FS et al. Front Endocrinol. (Lausanne). 2021;12:723750; 4. Saponaro F et al. Front Med (Lausanne). 2020;7:331.

Thyroid hormone exerts action through binding to nuclear thyroid hormone receptors:

Most thyroid hormone actions are initiated by binding of T3 to nuclear thyroid hormone receptors (TRα and TRβ)[1]

The monocarboxylate transporters, include MCT8 and MCT10, and are one of five protein families involved in **transport of T3 into cells**



Adapted from Galliford et al. (2023)[2]

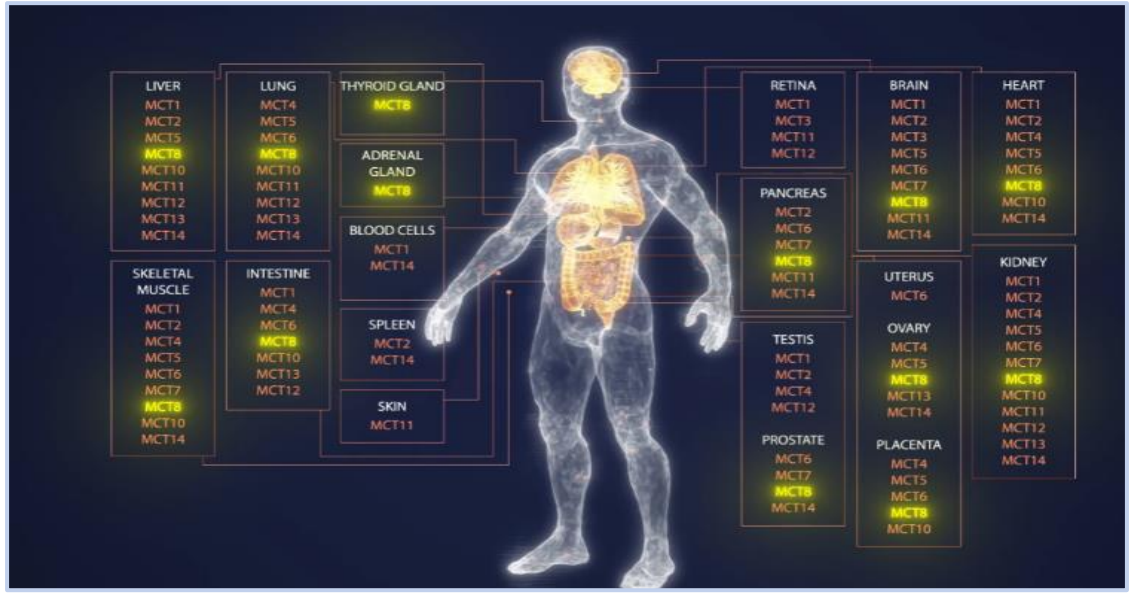
D2, type II iodothyronine deiodinase; D3, type III iodothyronine deiodinase; mRNA, messenger ribonucleic acid; RXR, retinoid X receptor; rT3, reverse T3; TR, thyroid receptor; TRE, thyroid response element; T2, diiodothyronine; T3, triiodothyronine; T4, thyroxine.

1. Saponaro F et al. Front Med (Lausanne). 2020;7:331; 2. Galliford T. (presentation) Available from: https://slideplayer.com/slide/4600022/. Accessed July 2023.

MCT8 is widely expressed in tissues:

Monocarboxylate transporter 8 (MCT8) is expressed in most tissues and is most prominently expressed in the liver, thyroid, kidneys, heart, lungs, and brain, facilitating:[1,2]

- Uptake of T3 and T4
- Efflux of T3 and T4



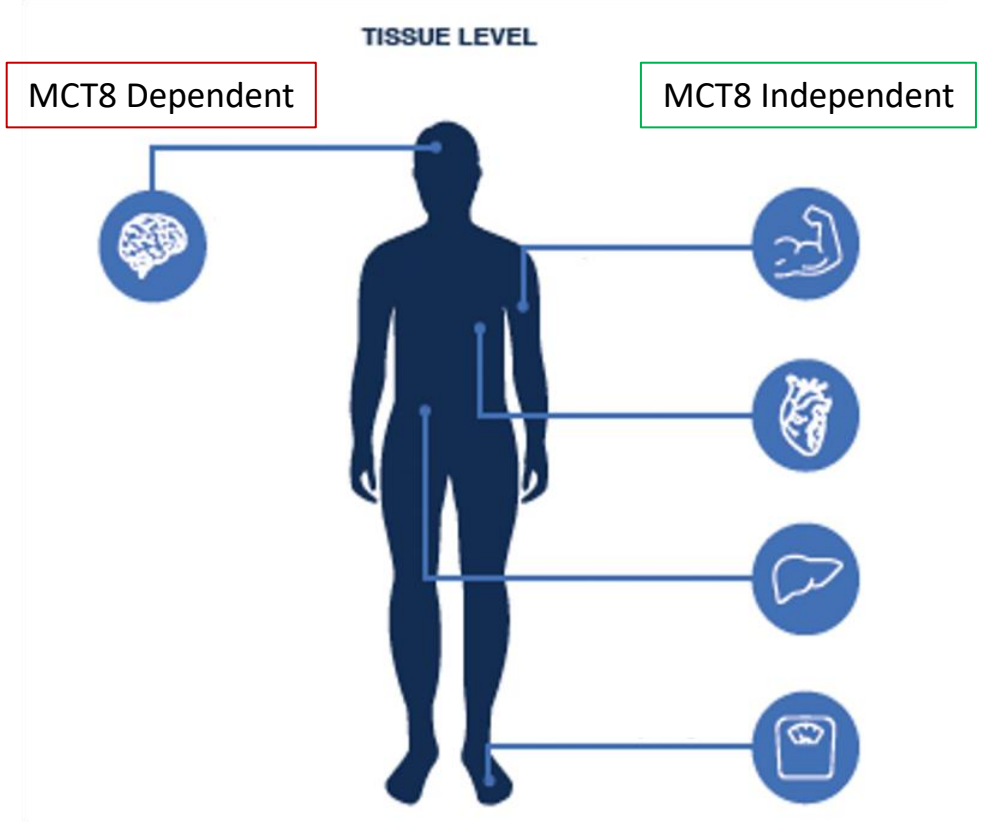
MCT8, monocarboxylate transporter 8; T2, diiodothyronine; T3, triiodothyronine; T4, thyroxine.

1. Groeneweg S et al. Endocr. Rev 2020;41(2):146–201; 2. van Geest FS et al. Endocrine. 2021;71:689–695.

*MCT8 is highly expressed in the liver, brain, kidney, heart, placenta, lung, and thyroid gland, as well as other sites including the pituitary gland[1,2]

MCT, monocarboxylate transporter; SLC16, solute carrier family 16; T3, Triiodothyronine; T4, thyroxine.

3. Felmlee MA, et al. Pharmacol Rev. 2020;72(2):466–485; 2. Groeneweg S et al. Endocr. Rev 2020;41(2):146–201.



Adapted from van Geest et al. (2021)[2]

MCT8 deficiency leads to a combination of low T3 in the CNS and elevated T3 in the periphery:

MCT8 deficiency leads to:[1,2]

Brain Hypothyroidism

Lack of thyroid hormone tri-iodothyronine (T3) in the CNS[2]

Body Hyperthyroidism

Chronic peripheral thyrotoxicosis[2]

Associated symptoms:

Absence of proper psychomotor development, developmental milestones not met[3]

Muscle wasting, decreased muscle mass, hypermetabolism of organs, sleep disturbance, weight loss, tachycardia and arrhythmia, and hypertension[1,4]

These factors contribute to the complexity of treatment

CNS, central nervous system; T3, triiodothyronine.

1. Sarret C, et al. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993–2023. Available from: https://www.ncbi.nlm.nih.gov/books/NBK26373/ Accessed July 2023; 2. Groeneweg S, et al. Lancet Diabetes Endocrinol. 2020;8(7):594–605; 3. NORD. MCT8-Specific Thyroid Hormone Cell Transporter Deficiency. Available from: https://rarediseases.org/rare-diseases/mct8-specific-thyroid-hormone-cell-transporter-deficiency/ Accessed July 2023; 4. Groeneweg S, et al. US Endocrinology, 2016;12(2):90–3.

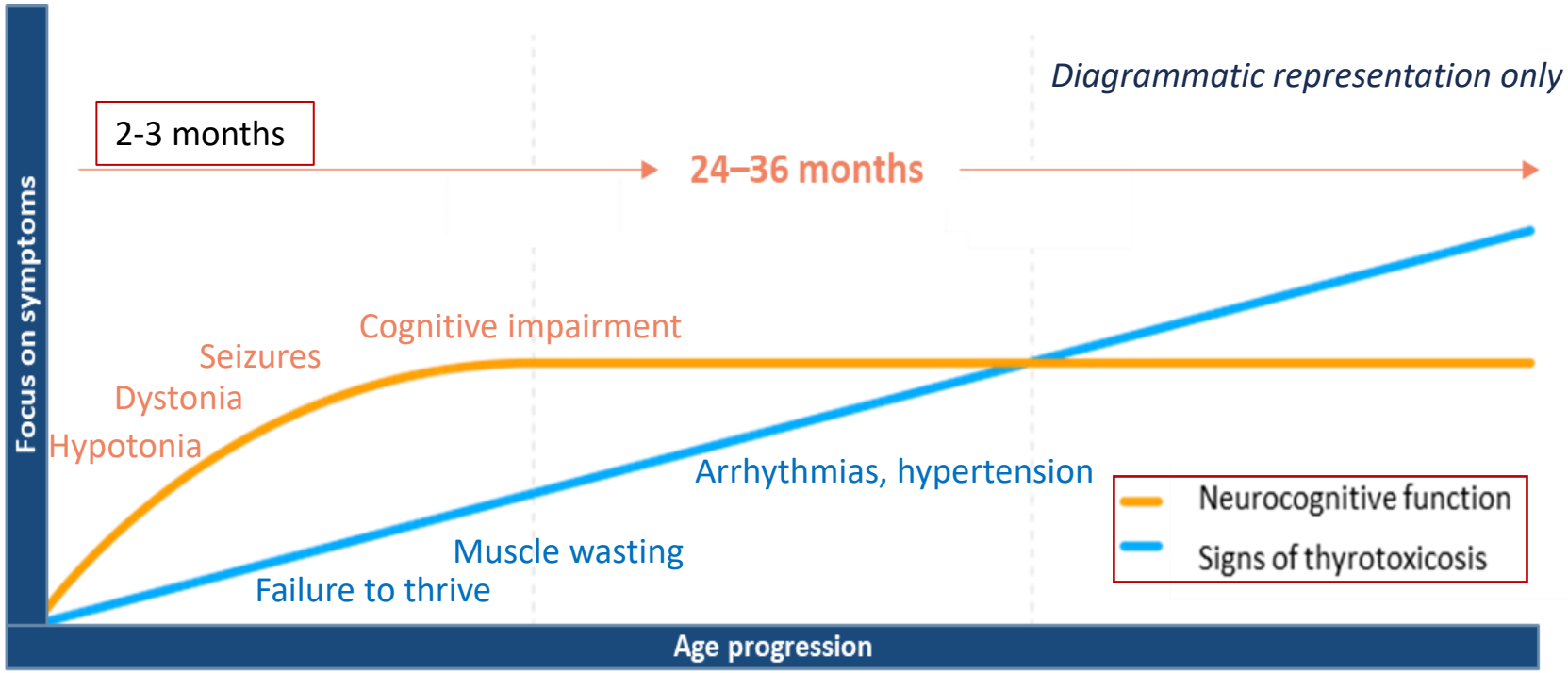
MCT8 deficiency - symptom evolution:

Schematic **representation of when specific symptoms become apparent over time[1]**

In the first few months, most affected infants do not display any symptoms

Parents report:[2]
- Pregnancy normal
- Apgar score normal
- Normal newborn screen

Newborn TFTs
- Normal -> low T4
- Normal TSH



1. Groeneweg S, et al. Lancet Diabetes Endocrinol. 2020;8(7):594–605; 2. Rodrigues F, et al. BMC Pediatr. 2014;14:252

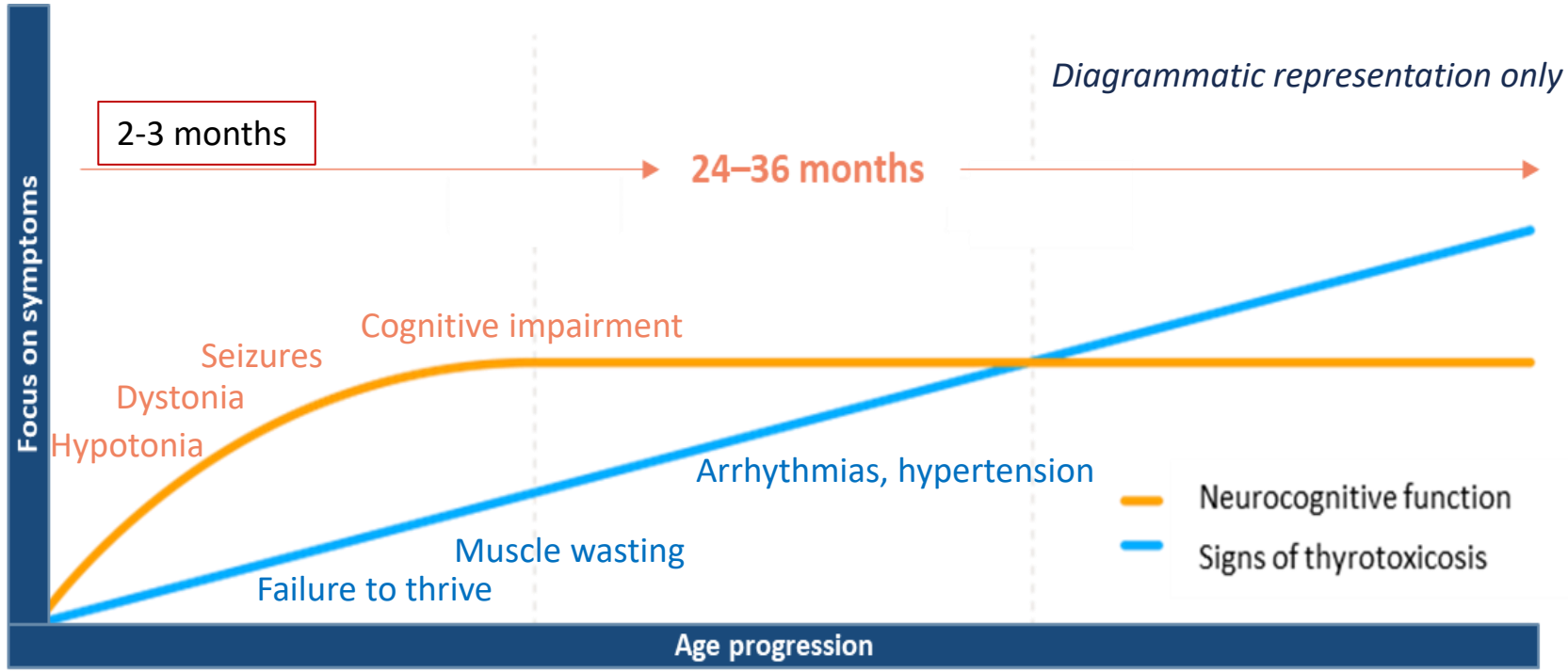
MCT8 deficiency - symptom evolution:

Schematic **representation of when specific symptoms become apparent over time[1]**

In the first few months, most affected infants do not display any symptoms

Parents report:[2]
- Pregnancy normal
- Apgar score normal
- Normal newborn screen

- **Loss of milestones around 3-6 months**
- **T3 increases**



1. Groeneweg S, et al. Lancet Diabetes Endocrinol. 2020;8(7):594–605; 2. Rodrigues F, et al. BMC Pediatr. 2014;14:252

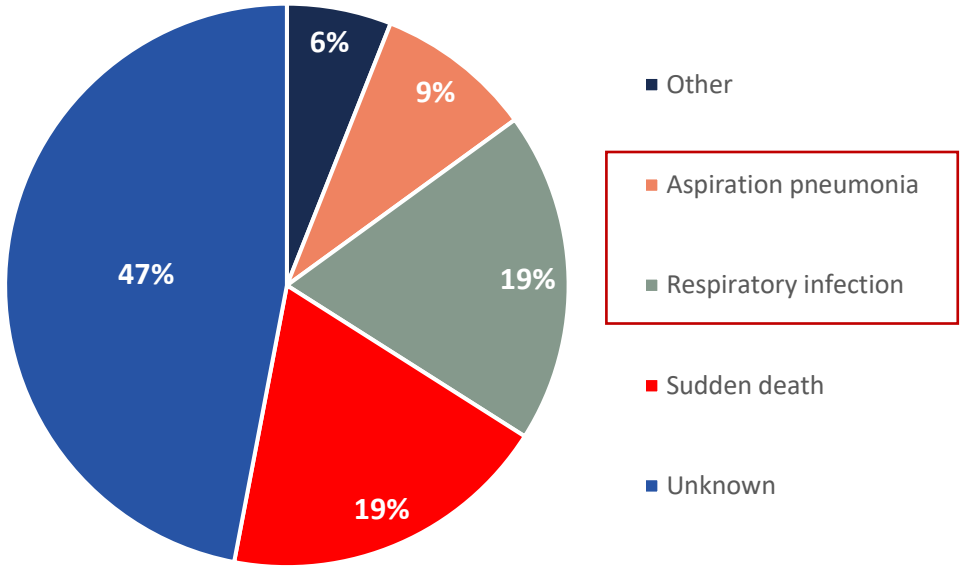
Living with MCT8 deficiency

Retrospective natural history study of 151 patients with MCT8 deficiency (Oct 2014–Jan 2020):[1]

- Median overall survival **35 years** (95% CI 8.3–61.7; n=145)
- 21% (32/151) of patients in this study died
- Median age at death **10.5 years** (range 1.6–71.0; n=32)

- Difficult, shortened life expectancy
  - Patient
  - Family/Care-giver

Causes of death in patients (n=32) with MCT8 deficiency†



†Amongst 32 patients who died at a median of 10.5 years old.

1. Groeneweg S, et al. Lancet Diabetes Endocrinol. 2020;8(7):594.

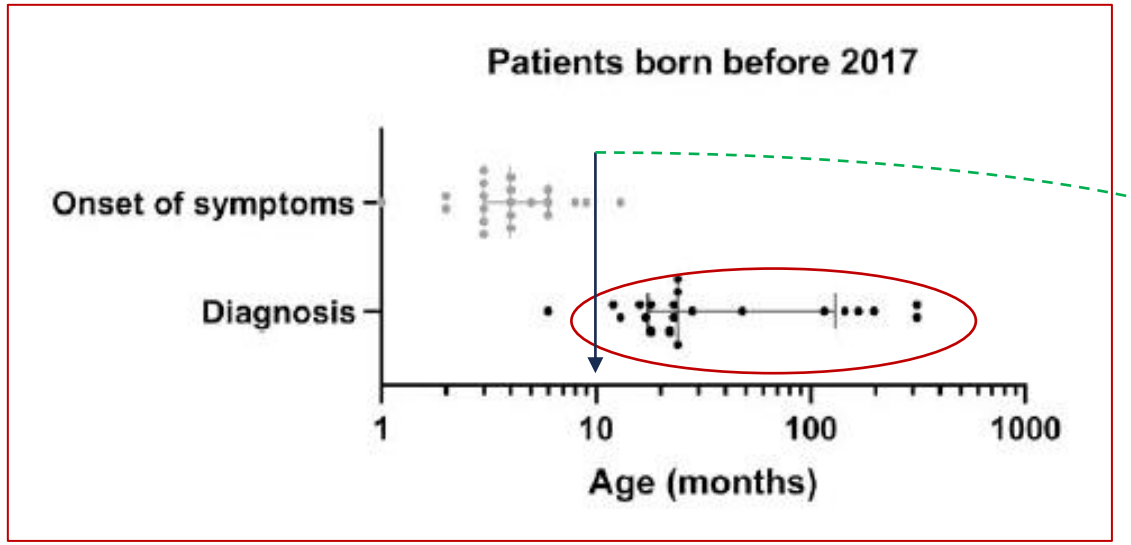
MCT8 deficiency is likely under-diagnosed:

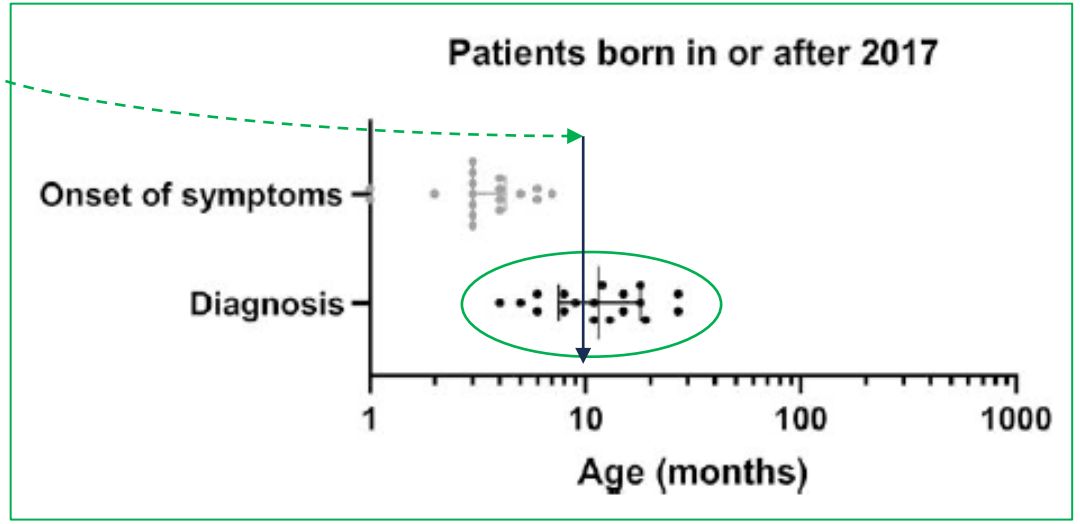
- **Newborn screening** is not designed to detect MCT8 deficiency.[1]
  - Improved understanding of disease, dynamic changes in thyroid hormone levels, access to LC-MS/MS and drug development are leading to discussion of strategies to improve earlier diagnosis
- The **pediatric neurologist** is often the first medical specialist to be seen.
  - Thyroid function testing often does not include **T3** or,
- **Knowledge and awareness of MCT8 Deficiency is poor, although improving,** in the medical community.
  - Underdiagnosed
  - Increased number of patients being diagnosed at an earlier age

T3, triiodothyronine.

1. Iwayama H, et al. Thyroid. 2021;31(9):1316–1321; 2. Dumitrescu AM, et al. Am. J. Hum. Genet. 2004;74;168–175; 3. Friesema EC. et al. Lancet 2004;364:1435–1437; 4. Zhang Q, et al. Frontiers in Pediatrics 2022;10:1050023; 5. Beheshti R, et al. Cureus. 2022;14(1):e21771

MCT8 deficiency is likely under-diagnosed:





1. van Geest H, et al. J Clin Endocrinol Metab 2023;31(9):11-6

MCT8 deficiency is likely to be under-diagnosed:

- **Newborn screening** is not designed to detect MCT8 deficiency.[1]
- The **pediatric neurologist** is often the first medical specialist to be seen.
- **Knowledge and awareness of MCT8 Deficiency is poor, although improving,** in the medical community.
- Improved genetic testing (multi-gene panels and WES) is identifying
  - More patients
  - New **pathogenic variants,** broadening our understanding of the spectrum of disease

T3, triiodothyronine.

1. Iwayama H, et al. Thyroid. 2021;31(9):1316–1321; 2. Dumitrescu AM, et al. Am. J. Hum. Genet. 2004;74;168–175; 3. Friesema EC. et al. Lancet 2004;364:1435–1437; 4. Zhang Q, et al. Frontiers in Pediatrics 2022;10:1050023; 5. Beheshti R, et al. Cureus. 2022;14(1):e21771

MCT8 deficiency - Summary

MCT8 deficiency is a rare and debilitating genetic disorder[1]

Dysfunctional activity of **monocarboxylate transporter 8** (MCT8) – a major transporter of thyroid hormone (T3, T4) into and out of cells[1,2]

X-linked genetic disorder = males

Symptoms **present early**, starting **in first 2–3 months** of life[1]

Characterized by **neurodevelopmental impairment** in combination with **signs and symptoms of thyrotoxicosis**[1,3-5]

MCT8 deficiency **profoundly impacts** long-term patient **health and the QoL of patients and carers**[3]

Current treatment options are limited to **supportive measures** which do not impact the underlying mechanism of disease

Unmet needs persist in **disease recognition, diagnosis, and treatment**[3,4,6–7]

QoL, quality of life; T3, triiodothyronine; T4, thyroxine.

1. Sarret C, et al. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993–2023. Available from: https://www.ncbi.nlm.nih.gov/books/NBK26373/ Accessed July 2023; 2. Groeneweg S, et al. 2016 US Endocrinology Available from: https://www.touchendocrinology.com/ Accessed July 2023; 3. CHOP. MCT8 Deficiency/Allan-Herndon Dudley Syndrome (AHDS). Available from: https://www.chop.edu Accessed July 2023; 4. Schwartz CE, Stevenson RE. Best Pract Res Clin Endocrinol Metab. 2007;21(2):307–21; 5. Groeneweg S, et al. Lancet Diabetes Endocrinol. 2020;8(7):594–605; 6. Rodrigues F, et al. BMC Pediatr. 2014;14:252; 7. Grijota-Martínez C, et al. Front Neurosci. 2020;14:380.

Current Treatments

Tiratricol is an investigational compound that is not approved by the FDA.
The safety and efficacy have not been established.

PTU* and LT4
(block and replace)

- ***PTU*** *– FDA Black Box Warning secondary to liver failure (2010)*

Tiratricol is an investigational compound that is not approved by the FDA.
The safety and efficacy have not been established.

Tiratricol

Background information on tiratricol is presented to help all participants fully align on the understanding of the disease.
A moderated discussion will follow and will provide time for questions and discussion

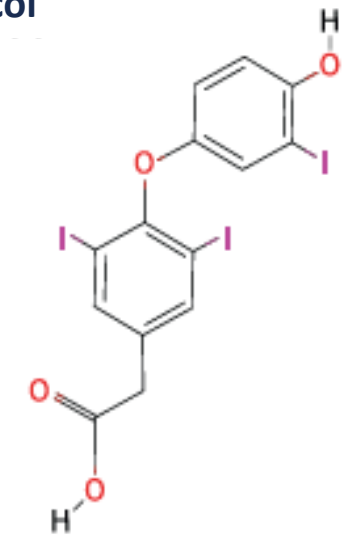
Tiratricol is an investigational compound that is not approved by the FDA.
The safety and efficacy have not been established.

Tiratricol is a bioactive metabolite of thyroid hormone:

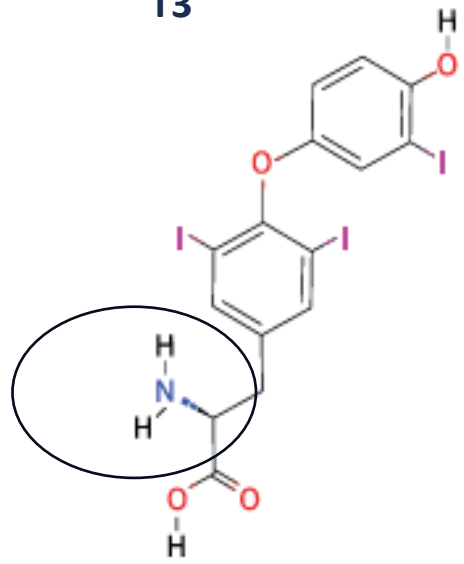
- Tiratricol is a bioactive metabolite of thyroid hormone (T3 and T4), present in low concentrations in the body[1,2]
- It has a **preserved iodination pattern** and similar signaling pattern to endogenous T3[1]
- Due to the **absence of the αNH2 group and one less carbon atom**, tiratricol can enter MCT8-dependent cells independent of functional MCT8 – bypassing the pathophysiologic defect in MCT8 deficiency[1,4,5]

Chemical structures of tiratricol and T3[2,3]

Tiratricol



T3



Images from Pubchem[2,3]

T3, triiodothyronine; T4, thyroxine.

1. Egetis. Emcitate Type C Meeting Package. Accessed July 2023; 2. PubChem. Available from: Tiratricol. https://pubchem.ncbi.nlm.nih.gov/compound/Tiratricol Accessed July 2023; 3. PubChem Liothyronine. Available from: https://pubchem.ncbi.nlm.nih.gov/compound/5920 Accessed July 2023; 4. Kersseboom S, et al. Mol Endocrinol. 2014;28(12):1961–1970; 5. van Geest FS, et al. Front Endocrinol (Lausanne). 2021;12:723750.

Tiratricol – overview of clinical studies:

Natural history[1]

- 2020
- Retrospective data (from 2003 – 2019)
- N=151

Triac Trial I[2]

- 2019
- Open-label, international, multicenter study
- N=46

Data Published

EMC cohort study[3]

- 2021
- N=67
- From Triac Trial I (N=27) and compassionate use program (N=40)

Triac Trial II[4]

- Started 2020 (LPO expected 2024)
- Open-label, international, multicenter
- Focus on neurodevelopment
- Patients age ≤ 30 months
- N=22

Re-TRIACt[5]

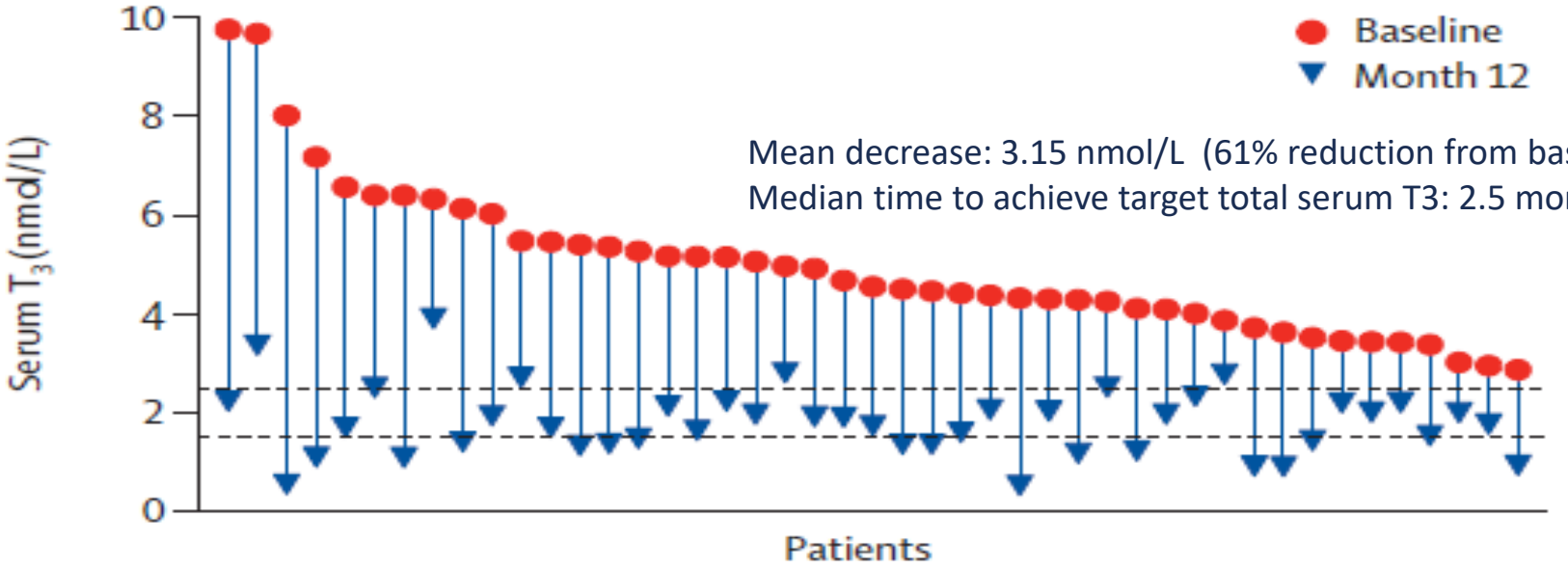
- Started 2023
- Prospective, double-blind, placebo-controlled data
- Patients age ≥ 4 yrs
- N=16

BA/BE, bioavailability/bioequivalence; LPO, last patient out.

1. Groeneweg S, et al. Lancet Diabetes Endocrinol. 2020;8(7):594–605; 2. Groeneweg S, et al. Lancet Diabetes Endocrinol. 2019;7(9):695–706; 3. van Geest FS, et al. J Clin Endocrinol Metab. 2022;107(3):e1136; 4. ClinicalTrials.gov. Available from: https://clinicaltrials.gov/study/NCT02396459 Accessed July 2023; 5. ClinicalTrials.gov. Available from: https://clinicaltrials.gov/study/NCT05579327 Accessed July 2023; 6. Personal communication, Egetis

TRIAC Trial I – primary endpoint of target serum T3

Changes in total serum T3 concentrations from baseline to 1 year per patient with MCT8 deficiency treated with tiratricol[1]



Mean decrease: 3.15 nmol/L (61% reduction from baseline)
Median time to achieve target total serum T3: 2.5 months

Endpoints	Baseline mean (SD)	12-month mean (SD)	Mean change (95% CI)	p-value
Primary (n=45)				
Total serum T3 (nmol/L)	4.97 (±1.55)	1.82 (±0.69)	-3.15 (-3.62, -2.68)	<0.0001
TSH (mU/L)	2.91 (±1.68)	1.02 (±1.14)	-1.89 (-2.39, -1.39)	<0.0001
Free T4 (pmol/L)	9.5 (±2.5)	3.4 (±1.6)	-6.1 (-6.8, -5.4)	<0.0001
Total T4 (nmol/L)	56.0 (±13.0)	24.4 (±9.4)	-31.6 (-35.2, -28.0)	<0.0001
rT3 (nmol/L)	0.12 (±0.10)	0.04 (±0.04)	-0.08 (-0.10, -0.05)	<0.0001

T3, triiodothyronine, CI, confidence interval; SD, standard deviation; TSH, thyroid-stimulating hormone

1. Groeneweg S, et al. Lancet Diabetes Endocrinol. 2019;7(9):695–706.

TRIAC Trial I – secondary efficacy outcomes:

Endpoints	Baseline mean (SD)	12-month mean (SD)	Mean change (95% CI)	p-value
Secondary (n=40)				
Weight for age Z score	-2.98 (±1.93)	-2.71 (±1.79)	0.27 (-0.03, -0.50)	0.03
Resting heart rate (bpm) (n=34)	112 (±23)	104 (±17)	-9 (-16, -2)	0.01
Mean heart rate over 24h (bpm) (n=31)	102 (14)	97 (±9)	-5 (-9, -1)	0.01
Blood pressure (n=32)	ND	ND	ND	ND
Systolic (mmHg)	108 (±8)	102 (±10)	-5 (-9, -1)	0.009
Systolic (percentile)	78 (±24)	61 (±29)	-18 (-29, -6)	0.004
Diastolic (mmHg)	64 (±9)	62 (±9)	-2 (-6, -2)	0.35
Diastolic (percentile)	74 (±22)	67 (±22)	-6 (-17, 4)	0.24
SHBG (nmol/L) (n=39)	212 (±91)	178 (±76)	-35 (-55, -15)	0.001
Total cholesterol (mmol/L)	3.2 (±0.7)	3.4 (±0.7)	0.2 (0.0, 0.3)	0.06
Creatine kinase (U/L)	108 (±90)	161 (±117)	53 (27, 78)	<0.0001

CI, confidence interval; SD, standard deviation; SHBG, sex hormone-binding globulin.

1. Groeneweg S, et al. Lancet Diabetes Endocrinol. 2019;7(9):695–706.

TRIAC Trial I – safety and tolerability:

Tiratricol was generally well-tolerated[1]

- 43 (93%) patients had at least one adverse event
- All seven adverse events that were suspected to be related to tiratricol treatment, which occurred in six (13%) patients, were mild
- Three patients had a transient increase in perspiration and three reported transient irritability. Both events resolved spontaneously after a few days
- **No patients required a dose reduction or discontinued participation because of drug-related toxicity**
- Most adverse events during the study period were classified as mild and required symptom relief or no treatment, and resolved while the patients continued to receive tiratricol
- No clinically relevant changes in cardiac structure or function were identified

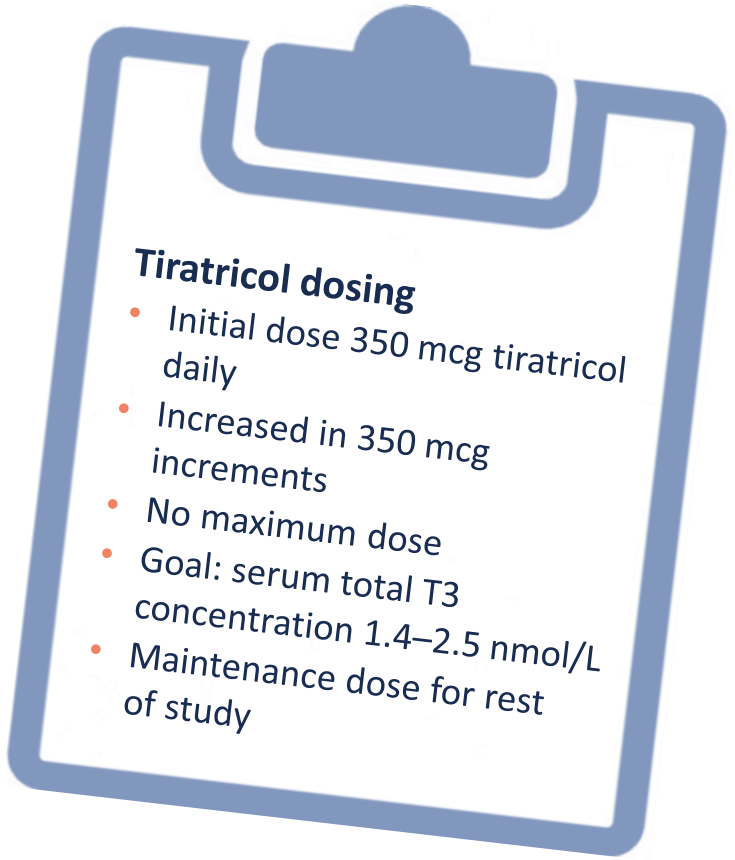
Adverse events occurring in >10% of patients	Patients with at least 1 event (N=46)	Number of events
Gastrointestinal disorders		
Diarrhoea	5 (11%)	5
Gastroenteritis	11 (24%)	12
Vomiting	5 (11%)	5
General disorders and administration-site conditions		
Influenza or influenza-like illness	9 (20%)	12
Infections and infestations		
Bronchitis	6 (13%)	6
Otitis media	5 (11%)	5
Respiratory, thoracic, and mediastinal disorders		
Nasopharyngitis	11 (24%)	14
Upper-respiratory-tract infection	9 (20%)	9

1. Groeneweg S, et al. Lancet Diabetes Endocrinol. 2019;7(9):695–706.

EMC cohort study (long-term data):

67 patients treated up to six years with tiratricol on compassionate or named patient use (NPU)

- Cohort consisted of
  - Patients who finished the TRIAC Trial I and continued treatment on a compassionate use program (n=27)
  - Patients who started treatment in a compassionate use program (n=40)
- 67 adult and pediatric patients treated up to 6 years
- Median baseline age 4.6 years (range 0.5–66 years)
- All patients received tiratricol orally daily with dose escalation to the same total serum T3 target range as in TRIAC Trial I

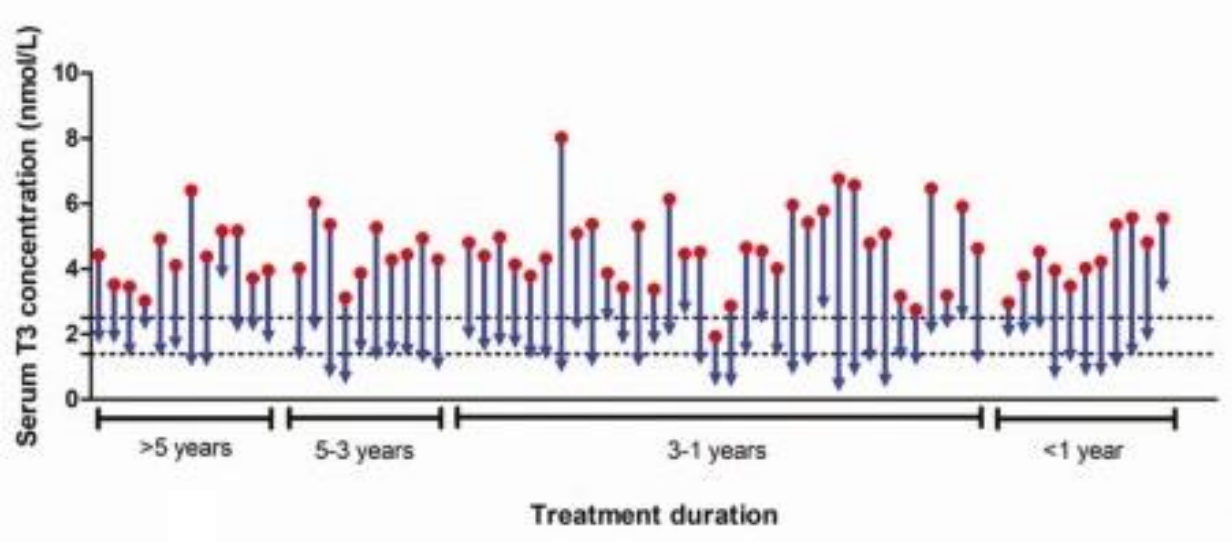


NPU, named patient use, T3, triiodothyronine.

1. van Geest FS, et al. J Clin Endocrinol Metab. 2022;107(3):e1136.

EMC cohort study (long-term data) – primary endpoint:

Changes in serum concentrations of T3 between baseline and last available follow-up visit on treatment with tiratricol, by patient, ordered by treatment duration



- At analysis, 67 patients treated for up to 6 years[1]
- **A fast* and sustained normalization of total serum T3 concentration was seen in almost all 67 patients regardless of patient age**
- Significantly reduced mean serum T3 concentration from baseline to last visit
  - Mean reduction: 2.92 nmol/L ($p<0.0001$)

*The daily dose was increased in increments with a goal of attaining serum total T3 concentrations within the target range of 1.4 to 2.5 nmol/L. The mean time to reach the maintenance dose was 5.0 (SD 4.7) months.
T3, triiodothyronine.

1. van Geest FS, et al. J Clin Endocrinol Metab. 2022;107(3):e1136.

EMC cohort study (long-term data) – safety and tolerability:

- No severe adverse events related to tiratricol were reported during follow-up[1]
- Transient signs of increased thyrotoxicosis were reported in a small subset of patients (5 of 67 patients)[1]
- The study data **substantiate the concept that tiratricol may be well-tolerated in the long term**[1]

Adverse events deemed related to tiratricol treatment[2]	Character
Increased irritability	Transient
Increased anxiety	Transient
Increased anxiety and sadness	Transient
Increased irritability and reduced sleep	Transient, after dose increase
Increased sweating and irritability, tachycardia	Transient
*Increased irritability and anxiety	Transient
*Increased blood pressure, tachycardia and increased anxiety	Transient, after dose increase

*These events occurred in the same patient

1. van Geest FS, et al. J Clin Endocrinol Metab. 2022;107(3):e1136; 2. van Geest FS, et al. J Clin Endocrinol Metab. 2021 Supplementary material. Available from: http://hdl.handle.net/1765/135544 Accessed July 2023.

Unmet Needs

Provider Perspective:

- **Prior to 2019 –> a devastating thyroid disease with no treatment**

1. van Geest FS, et al. J Clin Endocrinol Metab. 2022;107(3):e1136; 2. van Geest FS, et al. J Clin Endocrinol Metab. 2021 Supplementary material. Available from: http://hdl.handle.net/1765/135544 Accessed July 2023.

Provider Perspective:

- **Prior to 2019 –> a devastating thyroid disease with no treatment**

  - **Ideal targets of therapy**
    1. **Improve neurocognitive deficits**
    2. **Decrease peripheral hyperthyroidism**

Provider Perspective:

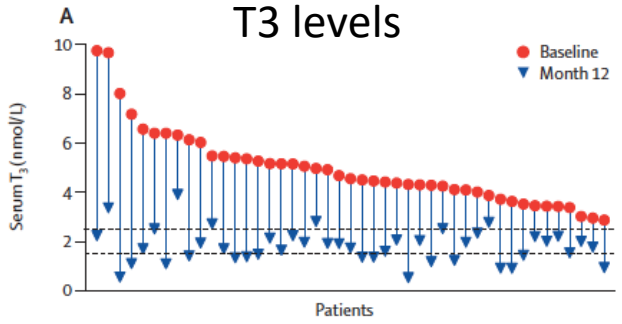
- **Prior to 2019 –> a devastating thyroid disease with no treatment**

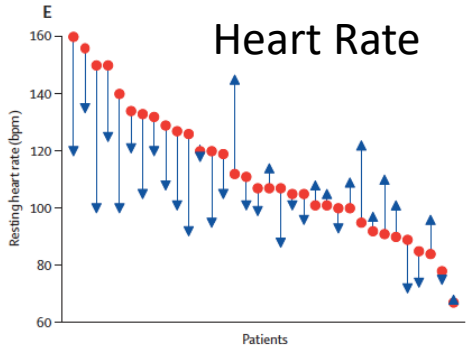
  - **Ideal targets of therapy**

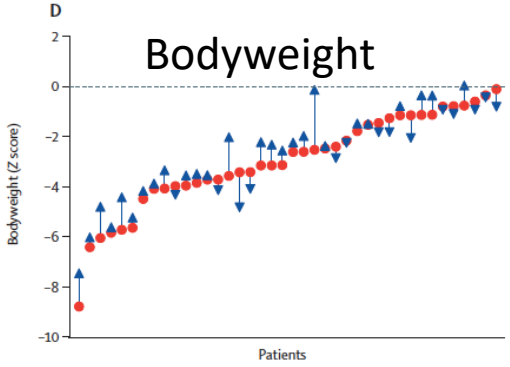
    1. **Improve neurocognitive deficits**
       - **Hypothyroidism associated damage to the brain**
         - **Starts during 1st trimester pregnancy**
         - **Permanent**
       - **Prevent further damage from time of diagnosis, ideally during time of neuroplasticity (< 3 years of age)**

Provider Perspective:

- **Prior to 2019 –> a devastating thyroid disease with no treatment**
- **2019**
  - **<u>75 years</u> after description of AHDS, and <u>60 years</u> after first use of tiratricol in clinical setting**



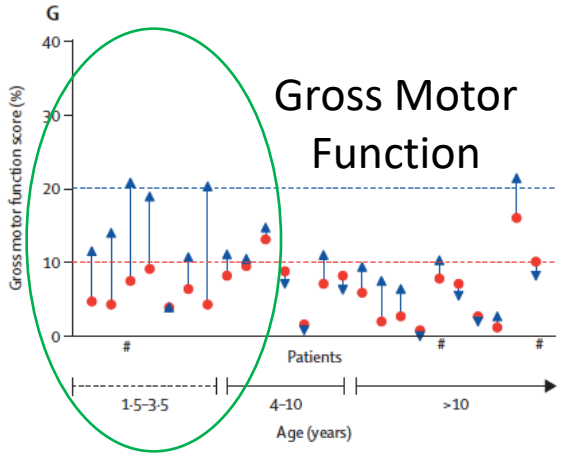




1. Groeneweg s, et al. Lancet Diabetes Endocrinol 2019;7(9):695-706 2. Bauer AJ, Lancet Diabetes Endocrinol, 7(9): 661-662.

Provider Perspective:

- **Prior to 2019 –> a devastating thyroid disease with no treatment**
- **2019**
  - **<u>75 years</u> after description of AHDS and <u>60 years</u> after first use of tiratricol in clinical setting**

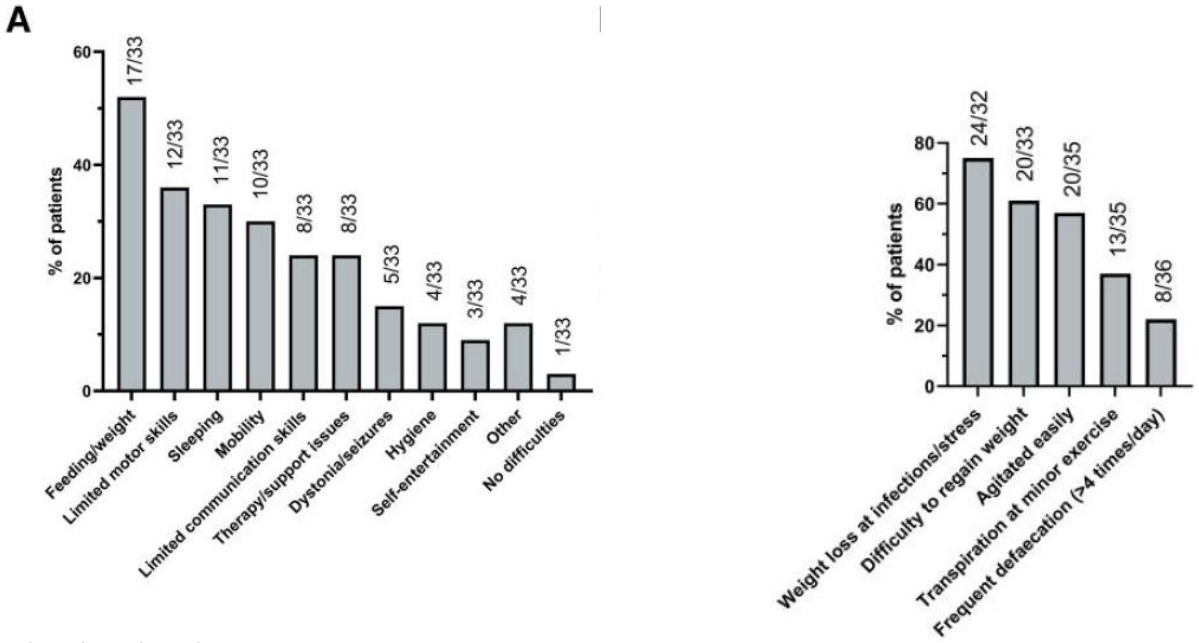


Phase II Trial
Patients < 30 months of age
Pending Results

1. Groeneweg s, et al. Lancet Diabetes Endocrinol 2019;7(9):695-706 2. Bauer AJ, Lancet Diabetes Endocrinol, 7(9): 661-662.

Parent Perspective:

- **2023** **Parent Perspectives on Complex Needs in Patients With MCT8 Deficiency: An International, Prospective, Registry Study** N = 36 ≤ 20 years



All parents (providers+) want to find solution to improve neurocognitive deficits

- Rx that improves living with peripheral hyperthyroidism is a significant step in the right direction

1. Van Geest et al, J Clin Endocrinol Metab 2023

Summary – current status of medical therapy

Checklist for clinical improvements using tiratricol

- ✓ **Decrease circulating levels of T3**
- ✓ **Decrease peripheral hyperthyroidism**
- ✓ **Low side-effects**
- ✓ **Parents voice high interest in continuing medication with increased awareness and seeking out Rx (observational)**

(-) no evidence that there is a cure to neurocognitive deficits

(?) improved neuromotor/neurocognitive function (pending data analysis)

1. Van Geest et al, J Clin Endocrinol Metab 2023

MCT8 Deficiency
Progress in improving
Unmet Needs for Patients and Care-Givers

Andrew J Bauer, MD

December 2023

WE CARE

FOR THE RARE

Egetis Investor Day 2023

December 19, 2023

Q&A

Dr Andrew Bauer & Nicklas Westerholm

EGETIS THERAPEUTICS

Global plans for commercializing Emcitate

December 19, 2023

Henrik Krook
VP Commercial Operations

Commercialization possible with lean & agile team

Unique setting for Emcitate in MCT8 deficiency

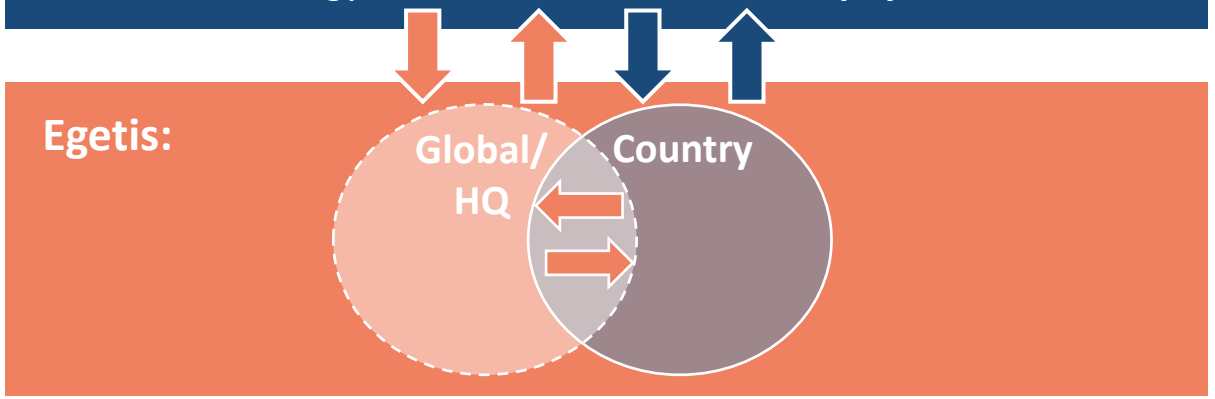
- Addressing unmet medical need
- No competition
- KOL support
- Centralized care guidance
- Global community

Seizing opportunity for cost-effective value creation

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

External Key Stakeholders:

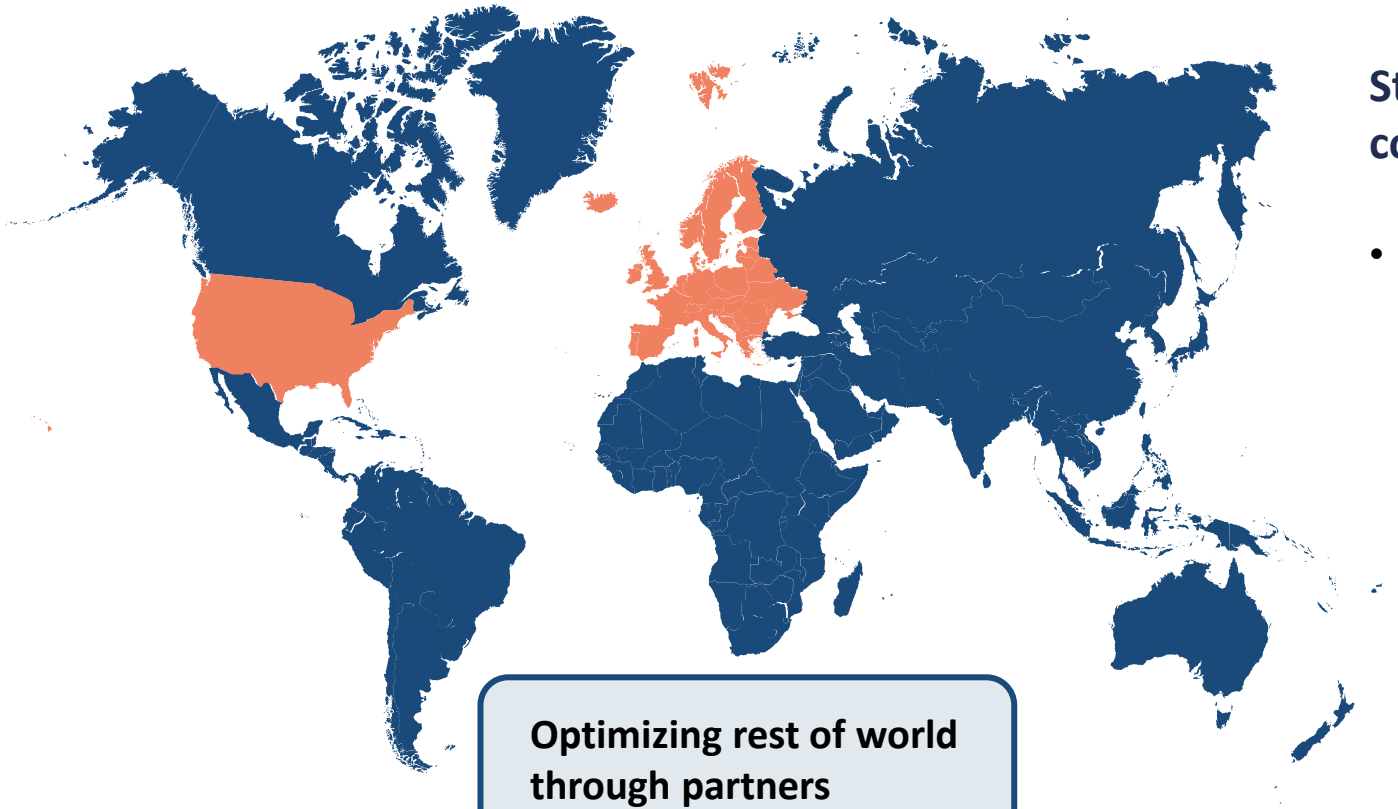
- **Caregivers** connected through international & national advocacy groups
- International **KOLs** & **physicians** at selected specialist centers
- Global strategy and local interactions with **payers**



Egetis commercialization of *Emcitate*

Retaining most value by managing the US & European 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 rest of world through partners

Stepwise establishing Egetis commercial capabilities

- Preparing for **launch** in US and Europe with an organization of **40-50** employees at time of launch

Building commercial organization to execute on key activities at the right time for launch success

Key projects driven by recognized industry talents recruited to the Egetis Commercial & Medical Affairs Team

– Leadership team brings launch skills and best practices from in total 100+ years at international companies

Henrik Krook, SE
VP, Commercial Operations

ALEXION

Marianne Berrens-Peijnenburg, NL
Global Head, Medical Affairs

SANOFI GENZYME

Nadia Georges, CH
Global Head,
Market Access & Pricing

SANOFI

Simon Rowe, UK
Global Business Excellence

Alnylam PHARMACEUTICALS

Peter Verwaijen, NL
Global Head Marketing & Brand
Strategy, GM Benelux & Iberia

ACTELION

Anny Bedard, US
President Egetis North America

SAREPTA THERAPEUTICS

Shire

Azza Trad, FR
GM France

sobi
Pioneer in Rare Diseases

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

BIOMARIN

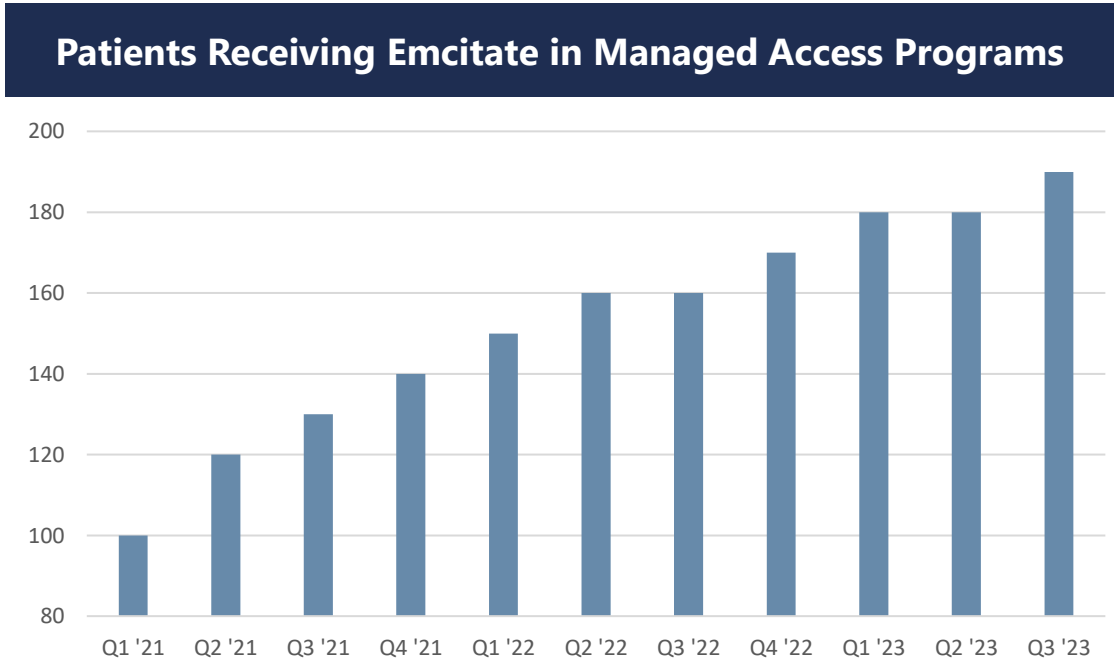
Raymond Francot, CH
GM for DE, AT, CH &
Central & Eastern Europe

VERTEX

Emcitate supplied globally in managed access programs

Continuous expansion confirms high unmet medical need

- 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
  - more than 190 patients
  - in over 25 countries



Aiming for broad access at optimal reimbursed price

LAUNCH VISION	*Everyone who can benefit from treatment, gets access to reimbursed Emcitate asap after regulatory approval*	
CRITICAL SUCCESS FACTORS	Fast and accurate diagnosis of affected patients	Payer support for reimbursement at optimal price
EXAMPLES OF KEY ENABLERS	Understand & address needs in MCT8 deficiency	Disease awareness & diagnosis initiatives Demonstrate & communicate *Emcitate* value

Payers in general accept higher prices for ultra-orphans

Opportunity for optimal reimbursed price

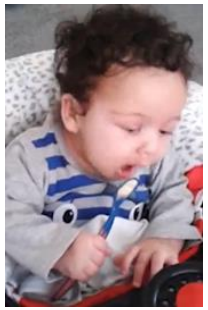
Payers in general **accept higher prices** for orphan drugs compared to traditional drugs and especially if they;

Emcitate fulfills these criteria

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

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

- A significant unmet medical need, addressed by the *Emcitate* clinical development program
- In addition, generating data for payers to answer the question *"What is the burden of MCT8 deficiency for patients, their caregivers & the healthcare?"*



Vignette study

Physician quantifying the impact of the disease on the patients' quality of life

Caregiver study

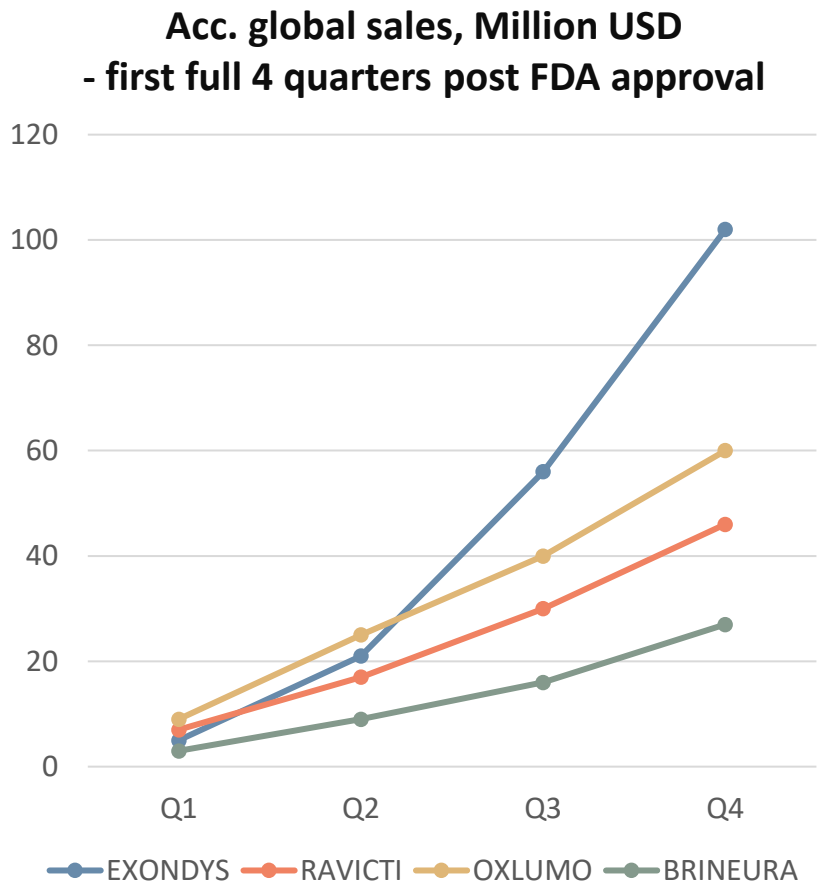
Focused on costs & quality of life for parents/caregivers

Healthcare resource use study

Describing healthcare resource use for the management of patients

Ultra-orphan analogues' sales uptake post approval

Several factors provide opportunity for swift uptake



Factors contributing to swift sales uptake for ultra-orphans

- Significant unmet medical need – lack of treatments
- No competition
- More patients being correctly diagnosed once approved drug available
- Payers reimburse at higher price levels
- Transfer of patients from managed access programs to commercial drug

EGETIS THERAPEUTICS

Understanding and meeting patient and caregiver needs

December 19, 2023

Nigel Nicholls
Global Head of Patient Advocacy
General Manager UK & Northern Europe

Egetis - A commitment to serve patients/caregivers

Ensure patients and caregivers are listened to as experts and their quality of life improved by our treatment

Egetis is listening and responding to caregiver needs

JOSH’S JOURNEY TO DIAGNOSIS

Hear what Josh’s mum noticed first and how diagnosis changed her family’s life and perspective.

Watch Josh’s story

Egetis is focused on minimising the diagnostic delay

- MCT8 patients are often undiagnosed or misdiagnosed
- Parents experience many months of uncertainty and frustration with profound impact on their quality of life

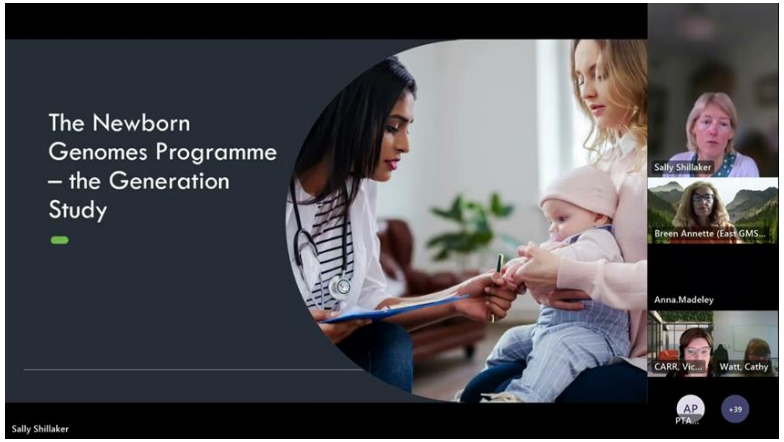
What Is Egetis doing?

Gene panel inclusion – SLC16A2 gene included in some important national gene panels

Patient caregiver study – Collating information for payors on impact to the family and society of MCT 8 deficiency

Patient Journey – Understanding paths to diagnosis and treatment to help to help accelerating time to diagnosis and establishing proper care for patient and family

Feeding tube study – Working with carers to understand the best way to administer *Emcitate*



Egetis is equipping & strengthening the patient/caregiver voice

- We are working and engaging with Patient Advocacy Groups across a range of important and strategic opportunities related to successful long-term reimbursement and access
- We are proud that caregivers helped in the design of the ReTRIACt study with home study nursing included as a result

LIFECYCLE MANAGEMENT

Pricing and Access

REIMBURSEMENT INVOLVEMENT

Patient voice and representation

AWARENESS / EDUCATION

Empowerment, stakeholder education and awareness

POLITICAL CAMPAIGNING AND ADVOCACY

'Fighting for access' Understanding and using media and political pathways

Egetis is building a larger and more impactful awareness voice

- Three interested and differing patient group communities are engaged in awareness raising

1. MCT8 deficiency focused

MCT8 - AHDS Foundation

SHERMAN FOUNDATION

MCT8 Forschung e.V.

Una Vita Rara
AHDS-MCT8 ONLUS

2. Thyroid conditions

Thyroid Federation International

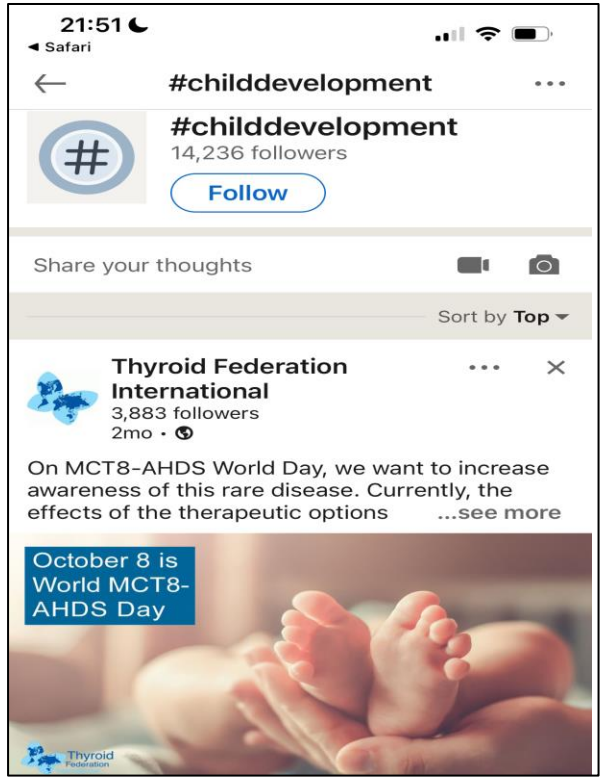
3. Rare disease

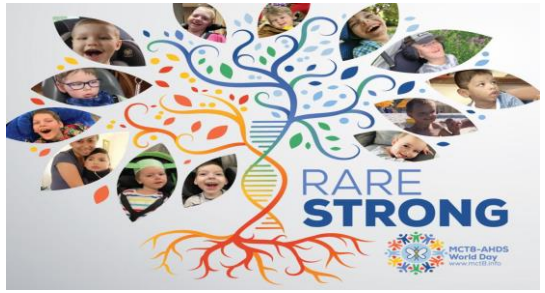
EURORDIS
RARE DISEASES EUROPE

NORD
National Organization for Rare Disorders

European Reference Networks
#ERNcare4Ua
Rare Diseases Doctors

Xtraordinaire.org
Handicaps mentaux liés au chromosome X
Association de familles





Egetis is Working Together for a Better Future for Patients and Caregivers

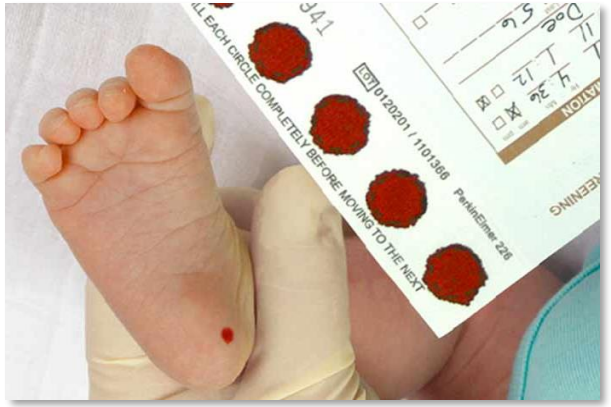
EGETIS THERAPEUTICS

Improving disease awareness of MCT8 deficiency

December 19, 2023

Peter Verwaijen
Global Head of Marketing & Brand Strategy
General Manager Iberia & Benelux

Building MCT8 deficiency awareness cost-effectively

PATIENT CENTRIC ENGAGEMENT MODEL	INSIGHT-DRIVEN CAMPAIGN	MAXIMISING CUSTOMER TOUCHPOINTS	PATIENT FINDING INITIATIVES
			

The most efficient way to increase awareness of an ultra-rare disease

Listen and learn to experts and families

- Advisory boards
- Market Research e.g. Patient journey mapping
- PAGs engagement
- Family & HCP interviews
- Local & international congresses

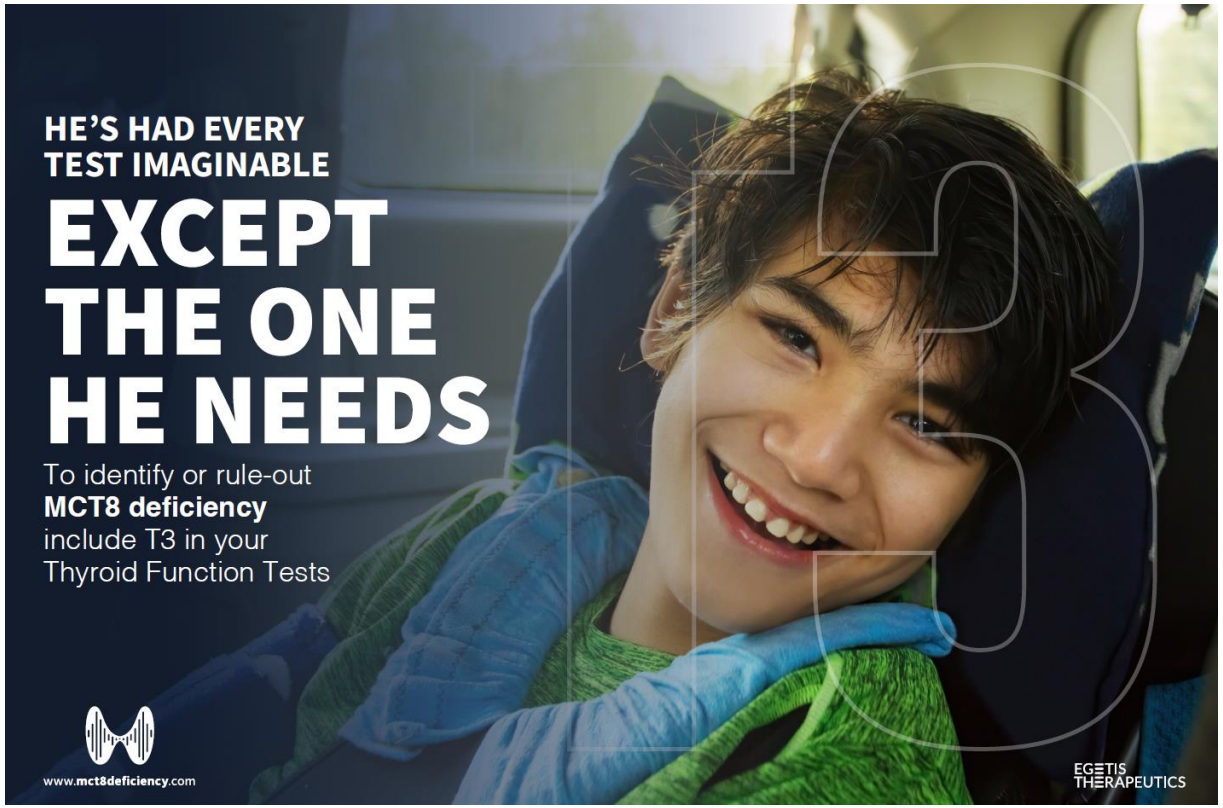
Support materials for HCPs and families

- Website
- Mode of disease video
- Social media
- Brochures
- Email campaigns
- Medscape eCME

PAG: Patient Advocacy Group
HCP: Health Care Professional
eCME: electronic Continuous Medical Education

Insight-driven creative and strategic messaging for HCPs

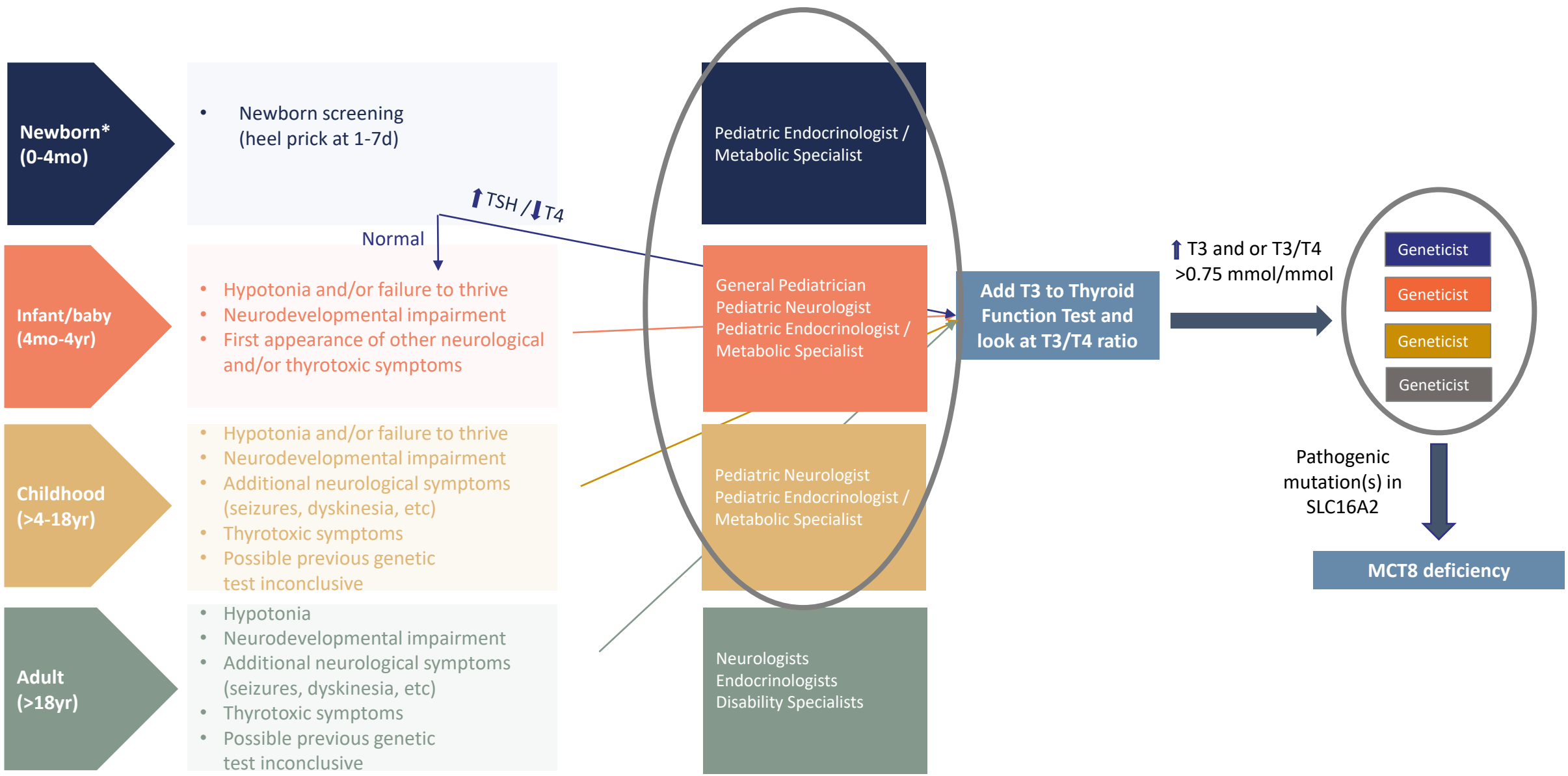
The One campaign



- The journey to an MCT8 deficiency diagnosis is almost always long and fraught with endless tests and doctor's appointments — often leading to dead ends
- The One campaign uses this insight to appeal to HCP's clinical and emotional interest in meeting an unmet need
- There is a clear, strategically-robust solution to the need: T3 testing, which guides critical success factors during pre-launch and launch phases
- MCT8 deficiency phenotype links to adding T3 to TFT where MCT8 deficiency is the motivator, followed by genetic confirmation

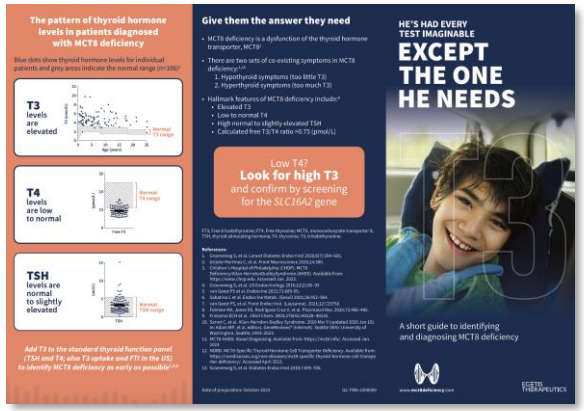
HCP: Health Care Professional
T3: active Thyroid Hormone
TFT: Thyroid Function Test

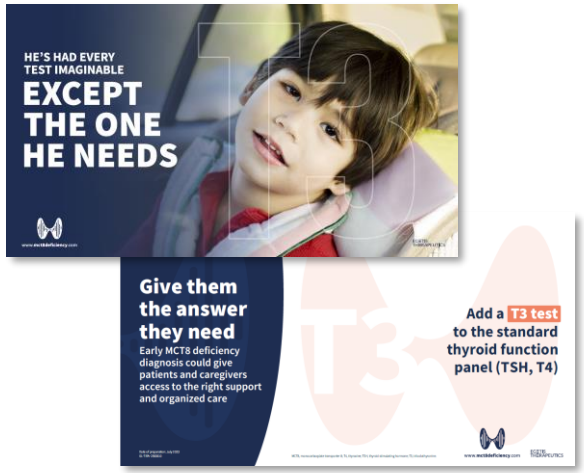
Target audiences are based on the patient journey



Deploying the campaign at key customer touchpoints

In 2023 Egetis attended 23 (inter)national congresses





Digital-first communications for public awareness and patient advocacy

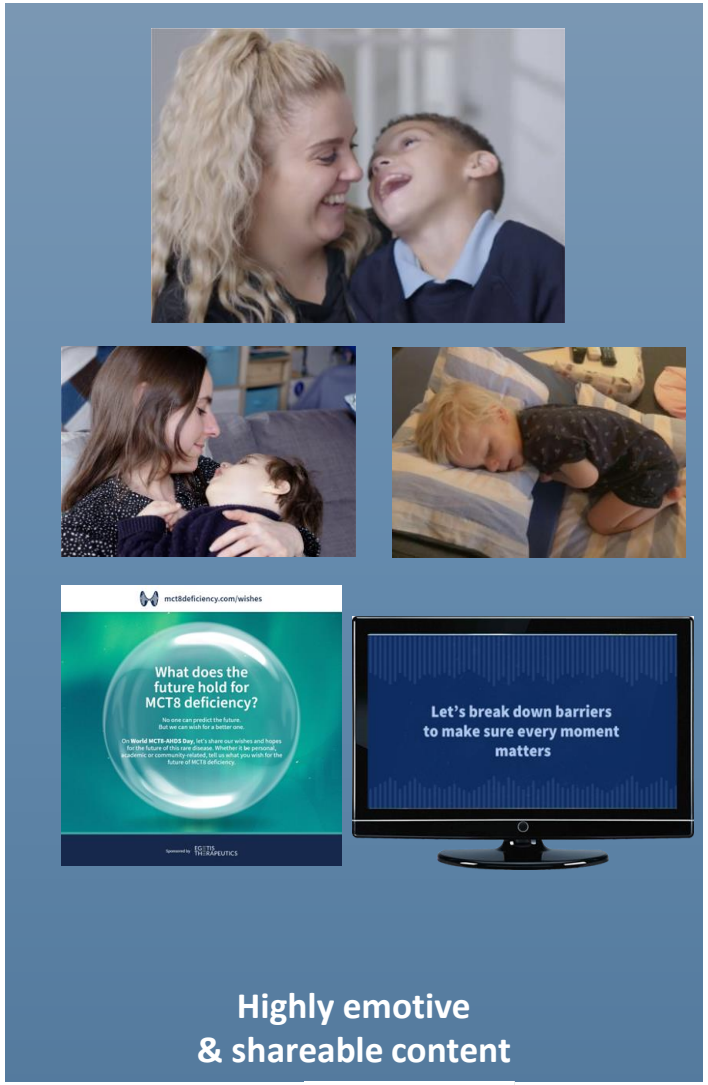
Disease awareness linked to awareness days & activities

RARE DISEASE DAY®

World Thyroid Day 25th May

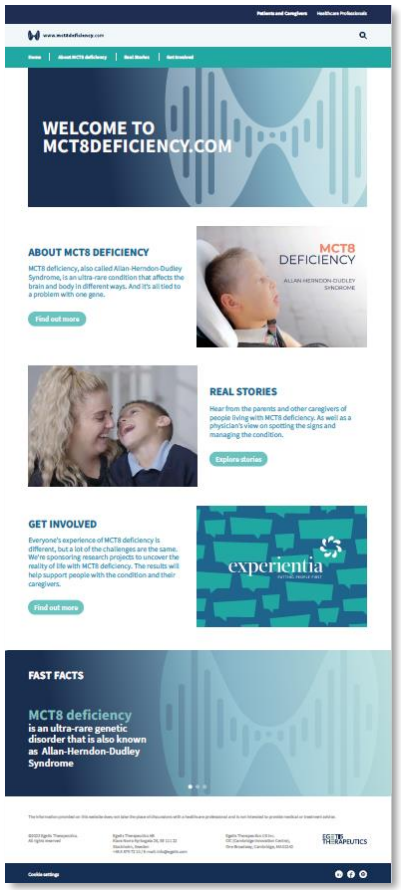
COLLABORATION WITH PAGS

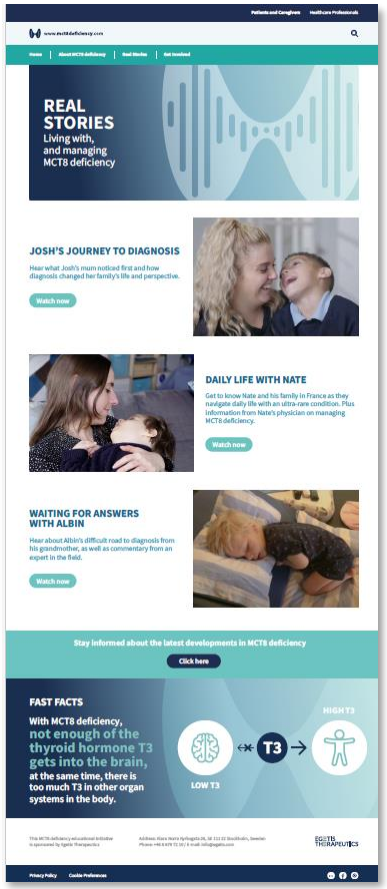
World MCT8-AHDS Day October 8th



Highly emotive & shareable content

All activities designed to increase disease awareness and drive traffic to mct8deficiency.com





mct8deficiency.com is our central disease awareness hub

CAREGIVERS

Internet search
Social media
Patient/caregiver materials
from HCPs /PAGs

INFORMATION SEEKING HCP

ADVOCACY GROUPS

CORE TOUCHPOINTS

GMS & FUTURE KAMS

Core story

Leave pieces

Veeva approved emailing campaign

CONGRESS & MEETINGS

Stand panels

Rich media

Unbranded literature

ADBOARDS

CME PROGRAMS

Continuing Medical Education Program
CME

Medscape

PeerVoice

WWW.MCT8DEFICIENCY.COM

Raise awareness of MCT8 deficiency

Raise awareness around value of spotting MCT8 deficiency and diagnosing it to ensure optimal care and management

Key assets housed:

- HCP/Patient/Caregiver videos
- Mode of disease video

Resource centre including:

- MoD animation
- HCP/Patient & Caregiver videos
- 'Get involved' with ongoing studies
- HCP section:
  - About MCT8 Deficiency
  - Diagnosing MCT8 Deficiency
  - Research & resources
    - Contact

ACTIVATED HCP

MEDIA & ONLINE

SOCIAL MEDIA (CONTINUOUS & LINKED TO AWARENESS DAY ACTIVITIES)

Infographic posts

Video led activity (UK & France HCP/ patient/caregiver videos)

SYMPTOMATIC SEARCH (ORGANIC & PAID)

Google search optimisation

Google ads

HCP MEDIA (PRINT & ONLINE)

European Thyroid Journal

EMJ European Medical Journal

EPG Health Media

Medscape

MCT8DEFICIENCY.COM branded resources with strong call to actions:

Caregivers – Spot the signs of MCT8 Deficiency

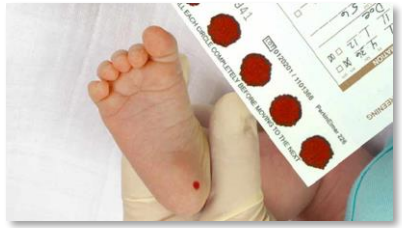
HCPs – Diagnosis is key: Include T3 in your TFT to rule-out/confirm MCT8 Deficiency

Patient finding initiatives

PATIENT SEGMENTS

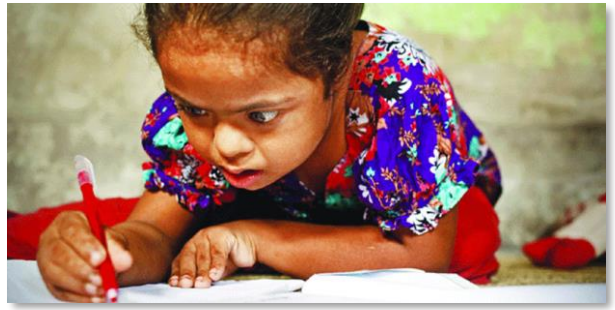
Newborn (0-4mo)

- Multiple touchpoints for possible diagnosis (after high TSH/low T4 on Newborn Screening (NBS) or through Newborn Sequencing (NBSequencing)



Infant/baby (4mo-4yr)

- Multiple touchpoints for possible diagnosis as symptoms begin appearing
- Caregivers and HCPs still highly motivated to continue the journey to diagnosis



Childhood (>4-18yr)

- Multiple touchpoints for possible diagnosis as symptoms worsening
- Potential genetic retesting and screening programs

Adult (>18yr)

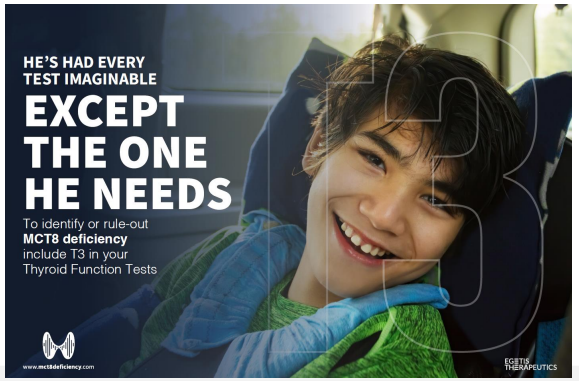
- Symptoms most likely treated with supportive care
- Potential genetic retesting and screening programs

Disease awareness initiatives are proving effective

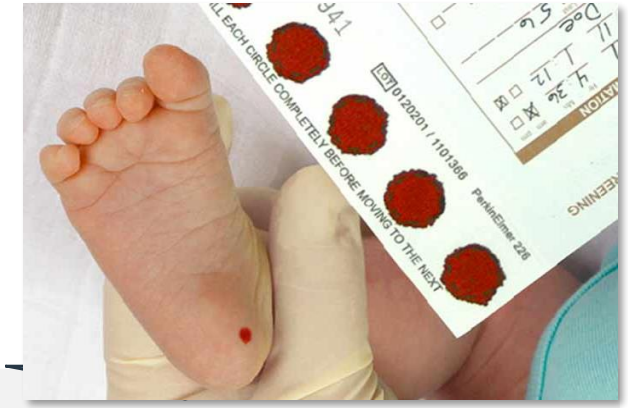
DISEASE AWARENESS AND EDUCATION

COLLABORATION WITH PAGs & KOLs

EXHIBIT AT SCIENTIFIC/MEDICAL CONFERENCES



PILOTS TO INCREASE DIAGNOSIS RATES



Increasing number of previously un- / misdiagnosed and treatment naïve patients are being identified

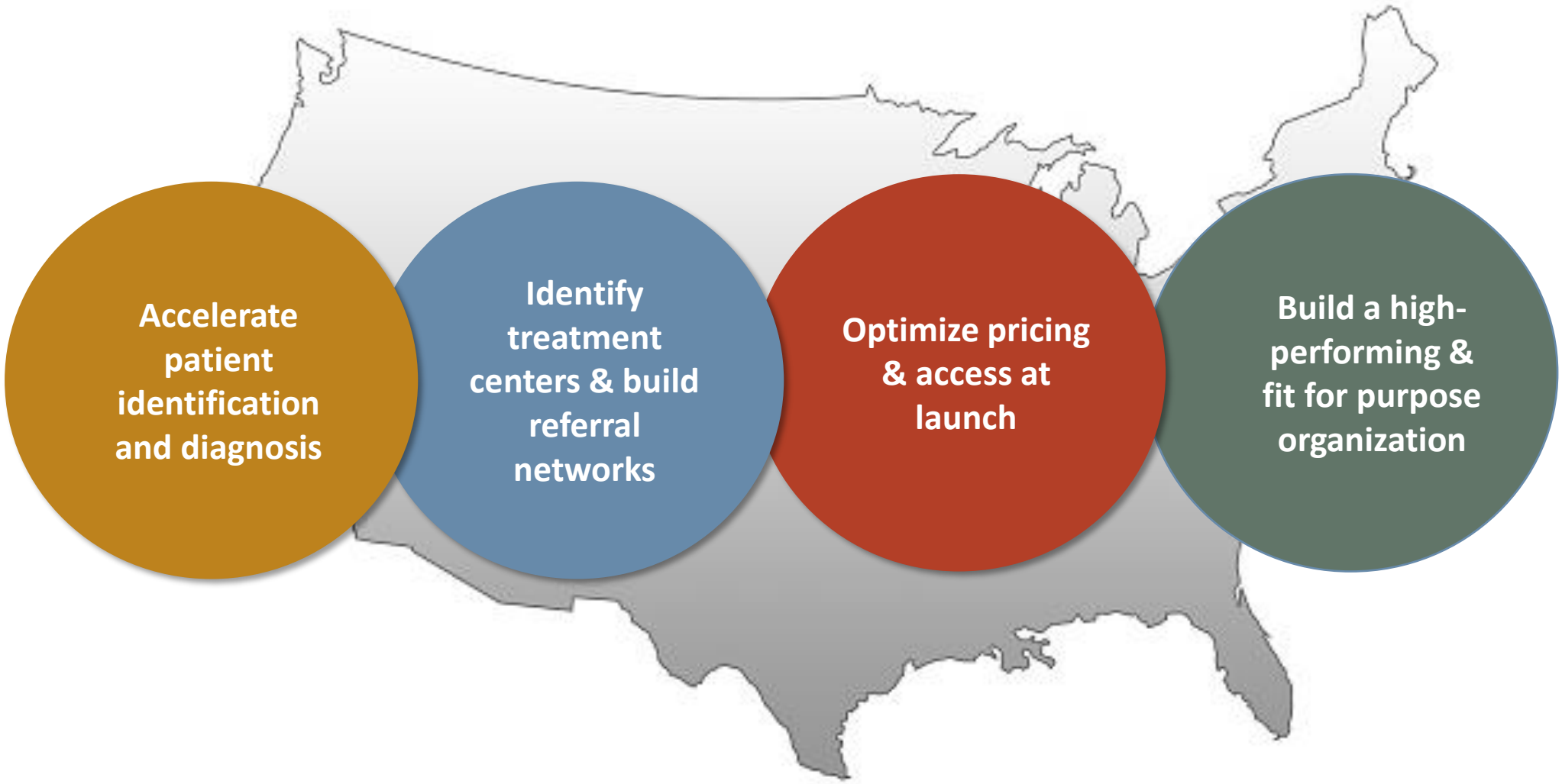
EGETIS THERAPEUTICS

US Launch Preparations for Emcitate

December 19, 2023

Anny Bedard
President Egetis North America

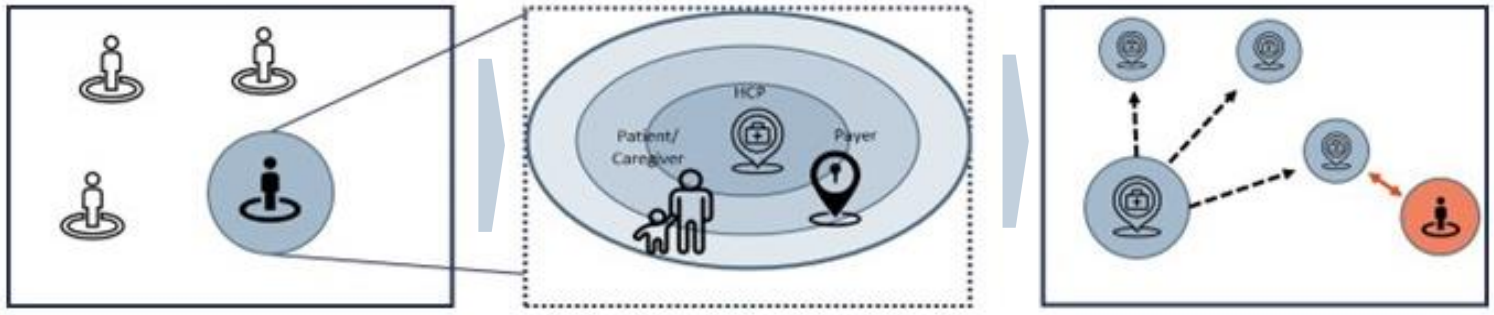
Key drivers shaping Egetis US commercial success



Effective deployment of our resources to enable patient finding

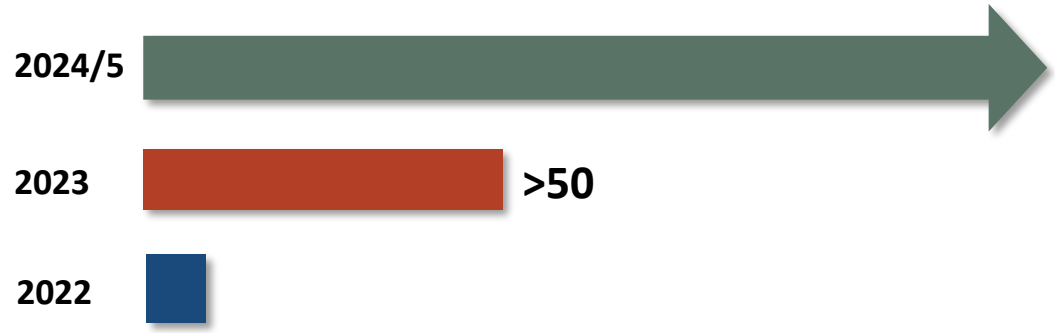
- Impactful presence at targeted scientific and medical conferences
- Quality engagements with HCP and patient advocates
- Partnership with high volume genetic testing & diagnostic service companies

Early HCP engagement strategy



Engage HCP with known patients | **Expand knowledge from the source** | **Create the "web of influence"**

Diagnosed MCT8 deficiency patients



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

Tiratricol (Emcitate) Expanded Access Program sites

https://www.clinicaltrials.gov/study/NCT05911399

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

Preparing for broad access at an optimized price

Analogue selection criteria

- ✓ Rarity (ultra-orphan)
- ✓ Paediatric
- ✓ No treatment options
- ✓ Life-long treatment
- ✓ Life threatening, debilitating

US payer analogues

Product	Disease	US gross annual treatment cost
Exondys® anti-sense oligonucleotide	Duchenne Muscular Dystrophy (13% of population)	$750k
Ravicti® small molecule	Urea Cycle Disorders	$750K
Oxlumo® iRNA	Primary Hyperoxaluria	$500k
Brineura® recombinant enzyme	CLN2	$750k

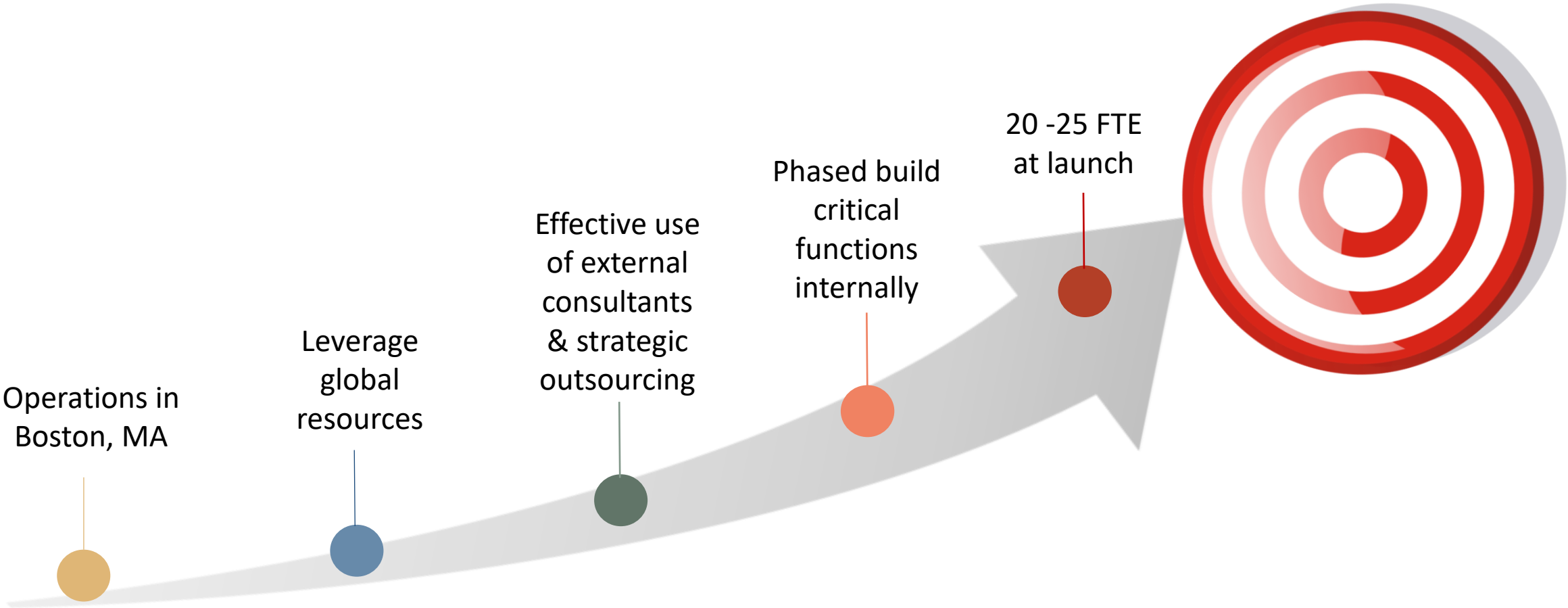
Most MCT8 deficiency patients will be eligible for government insurance

- Predominantly government payer mix

Exclusive distribution model through specialty pharmacy

- Expertise in negotiating with insurers
- Personalized patient access assistance
- Maintaining control of net price

Stepwise built and fit for purpose organization to deliver on the opportunity



Realizing the opportunity for succesful launch of Emcitate

- Disease awareness activities already bearing fruits
- Continuous expansion of the *Emcitate* Managed Access Program confirms high unmet medical need
- Payers reimburse at higher price levels in ultra-orphan setting
- Execution by nimble team of 40-50 employees in the US and Europe at the time of launch
- Analogues exhibit swift sales uptake post marketing authorization

WE CARE

FOR THE RARE

Egetis Investor Day 2023

December 19, 2023

Q&A

Henrik Krook, Nigel Nicholls, Peter Verwaijen, Anny Bedard & Nicklas Westerholm

WE CARE

FOR THE RARE

Egetis Investor Day 2023

December 19, 2023

BREAK

Resistance to Thyroid Hormone β and the Unmet Medical need

Dr Carla Moran

Consultant Endocrinologist

Beacon Hospital & St Vincent's University Hospital, Dublin

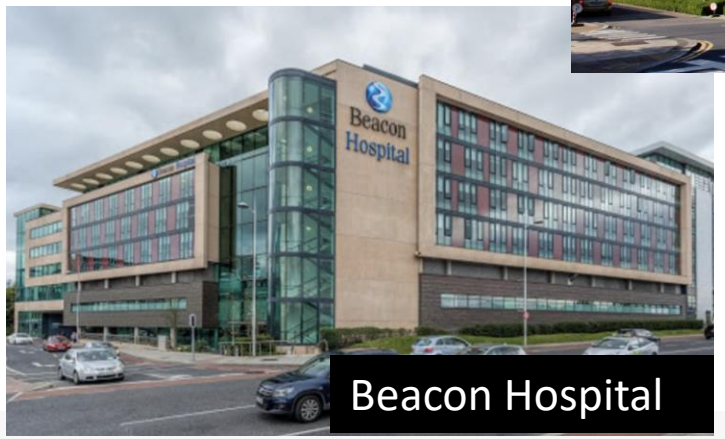
Associate Professor, University College Dublin

19th December 2023

Resistance to Thyroid Hormone BETA
=
RTHβ

Background

Cambridge University &
Cambridge University Hospital



Beacon Hospital

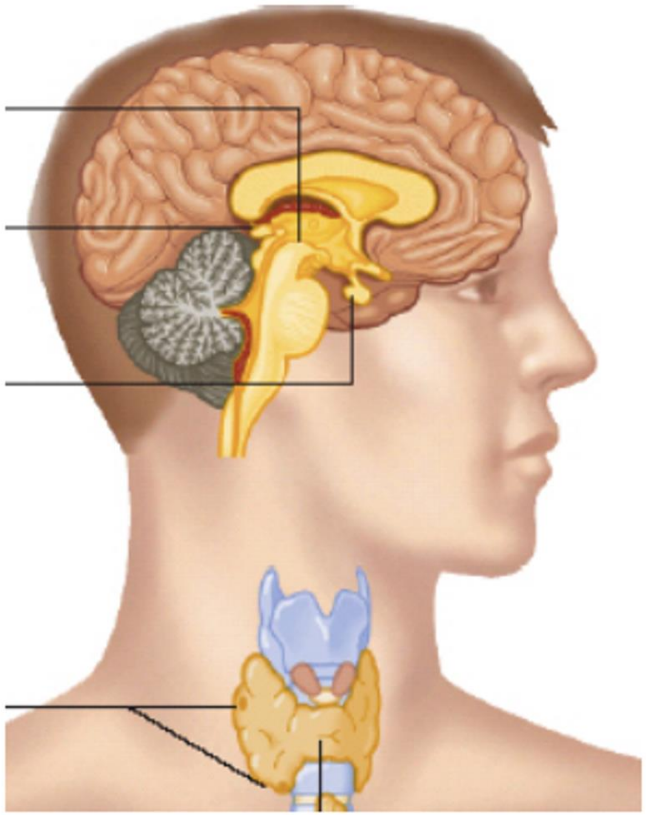
Overview

1. Overview of Thyroid Hormone
2. What is Resistance to Thyroid Hormone β?
3. Diagnosis of Resistance to Thyroid Hormone β
4. Effects of Resistance to Thyroid Hormone β
5. Treatment options, unmet needs
6. Future research priorities

Overview

1. Overview of Thyroid Hormone

Thyroid Hormone Production



Thyroid Stimulating Hormone (TSH)

+

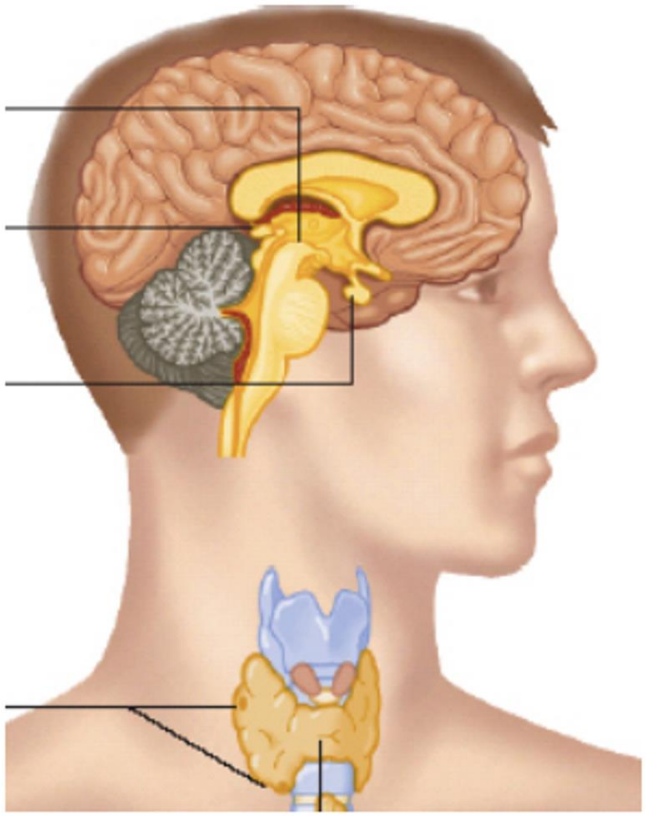
Thyroid Hormones
T4 and T3

Thyroid Hormone Production: “The Feedback Loop”

Pituitary

Thyroid

-

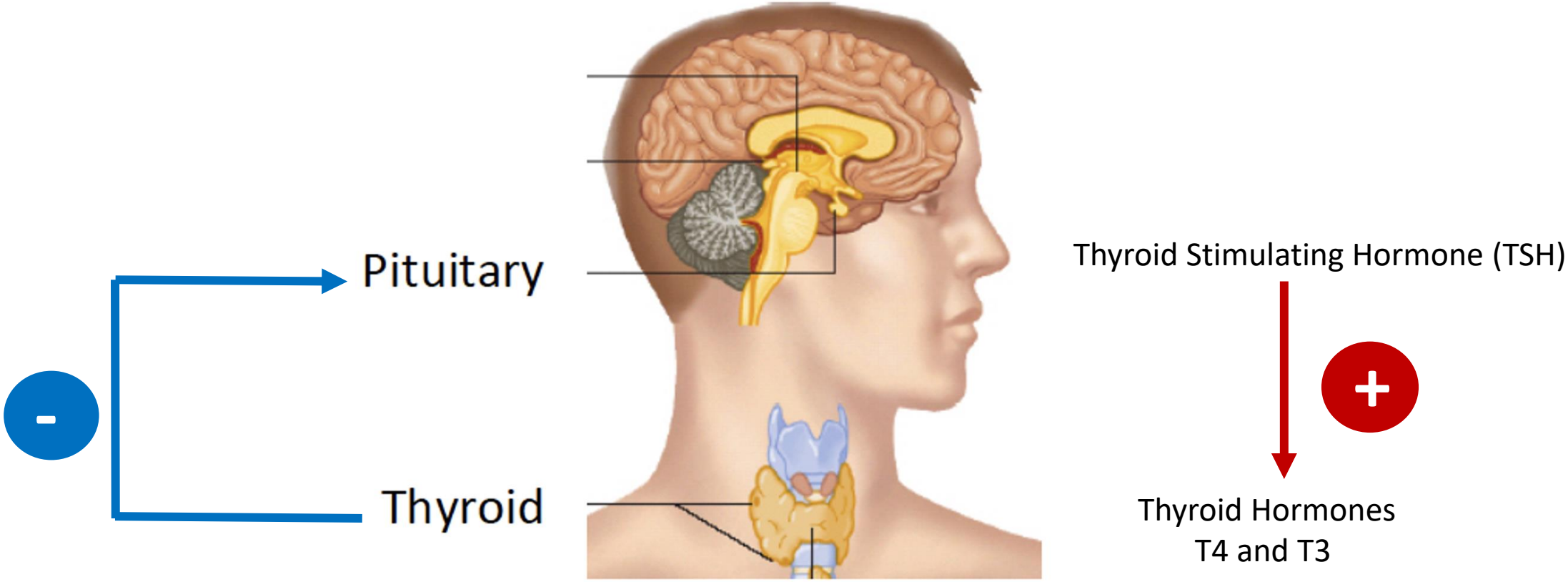


Thyroid Stimulating Hormone (TSH)

+

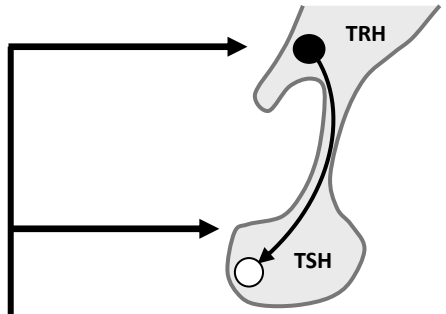
Thyroid Hormones
T4 and T3

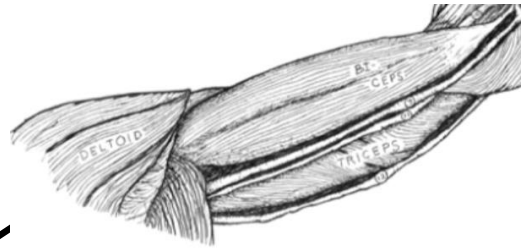
Thyroid Hormone Production: “The Feedback Loop” Example



	Overactive Thyroid Gland	Example levels	Normal Levels
TSH	LOW	<0.03	0.27-4.2
T4	HIGH	45	12-22
T3	HIGH	22	3.1-6.8

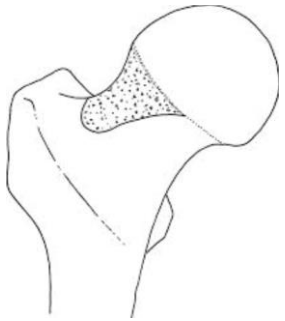
Normal Thyroid Hormone action





Muscle

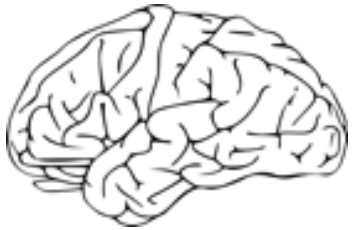
Metabolic Rate



Bone

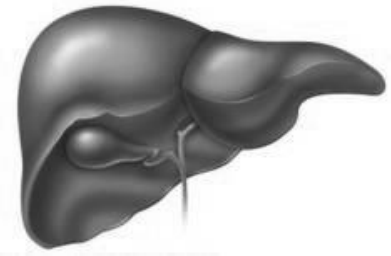
Bone maturation
Bone turnover
Growth

T3 T4



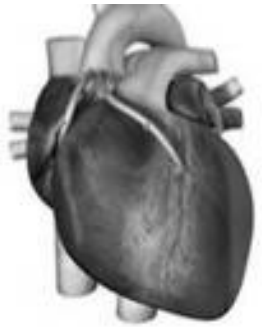
CNS

Brain development
Myelination



Liver

SHBG production
Cholesterol metabolism



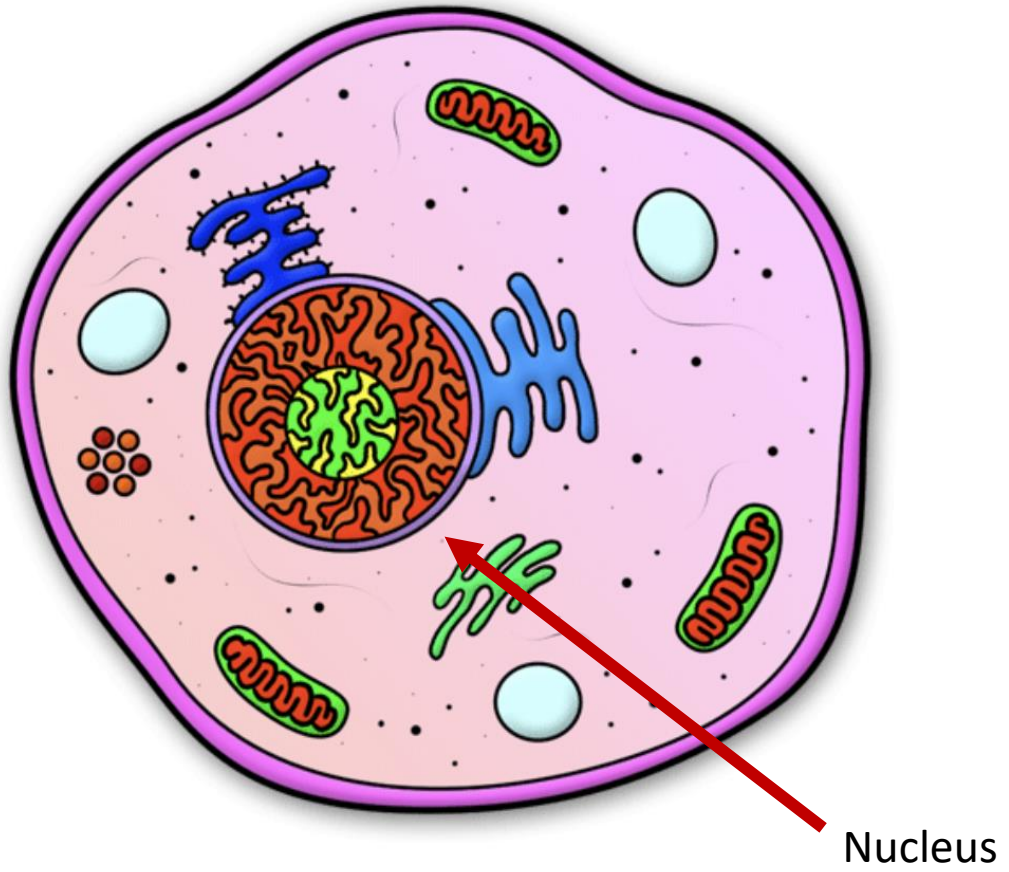
Heart

Inotropic effects
Chronotropic effects

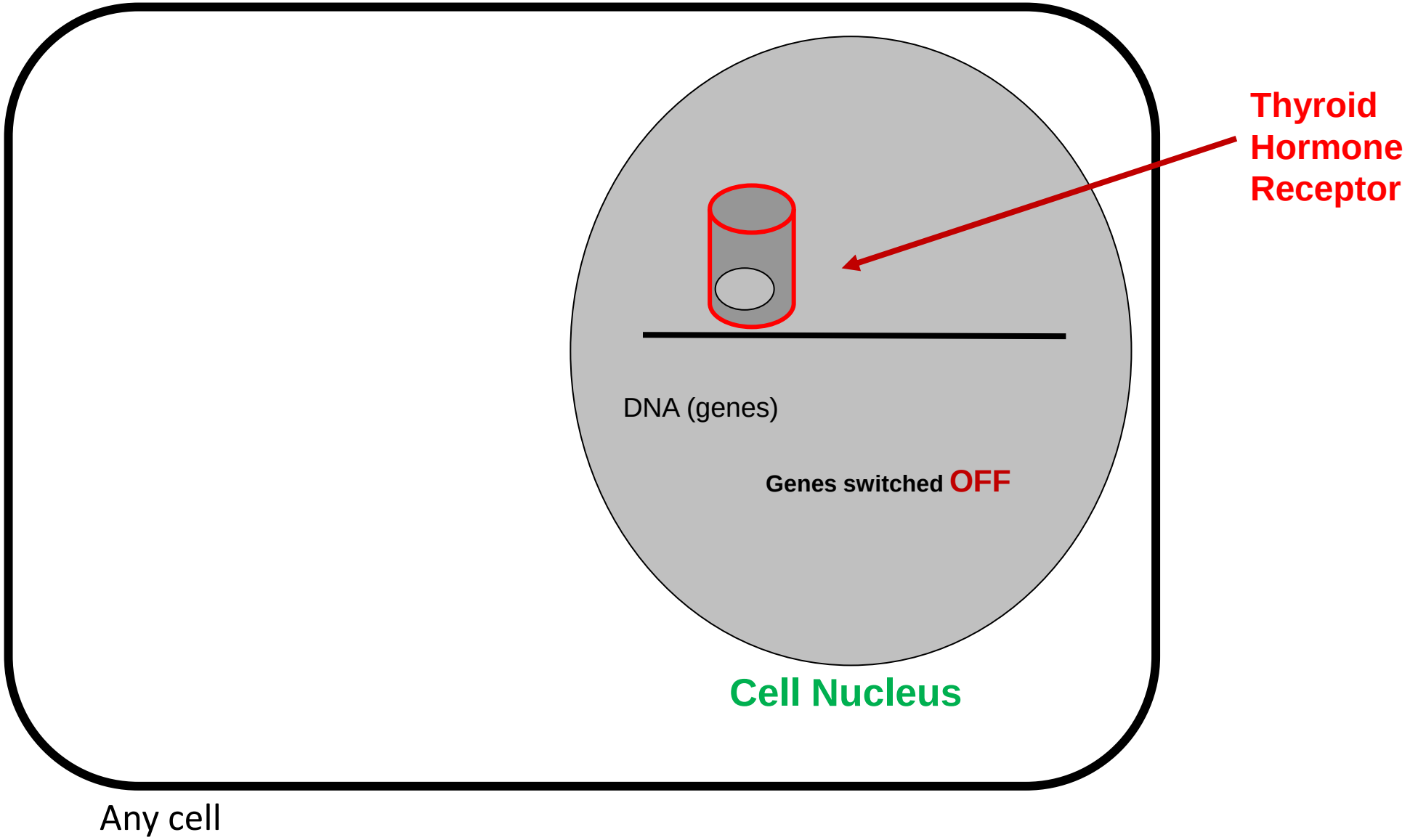
Adapted from Moran C. *Best Pract Res Clin Endocrinol Metab.* 2015. PMID 26303090

But how do Thyroid Hormones actually work?

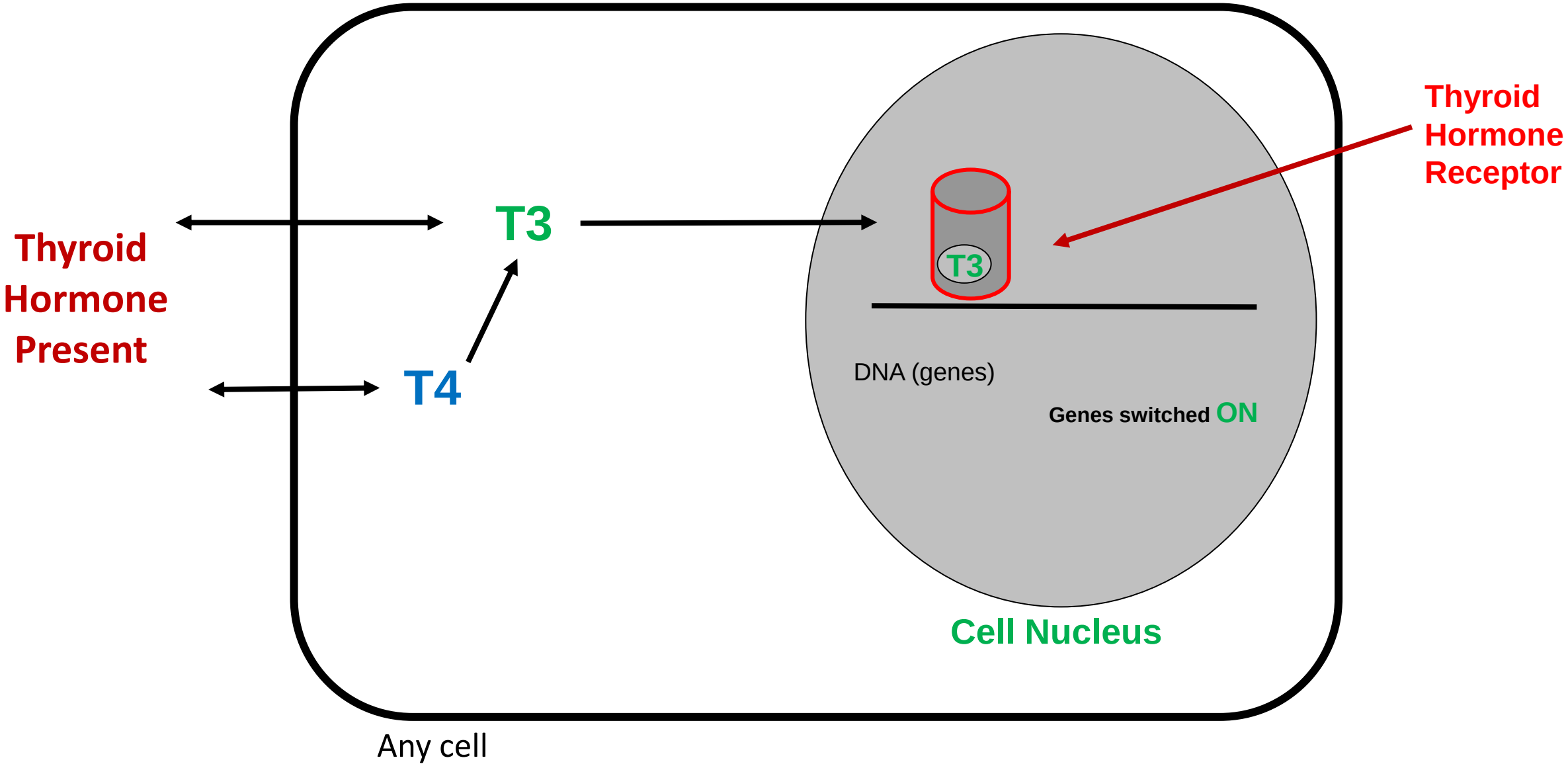
To answer this we need to go inside the cell



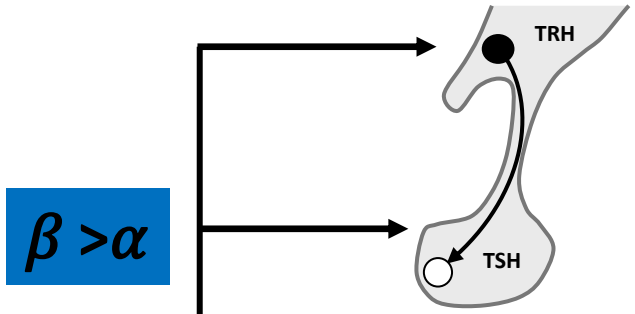
But how do Thyroid Hormones actually work?



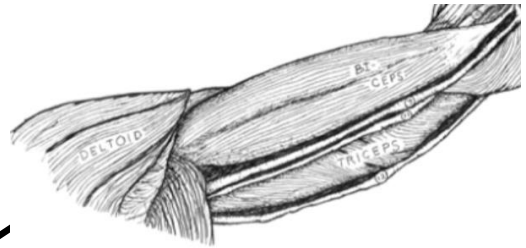
But how do Thyroid Hormones actually work?



Thyroid Hormone Receptors – TWO FORMS



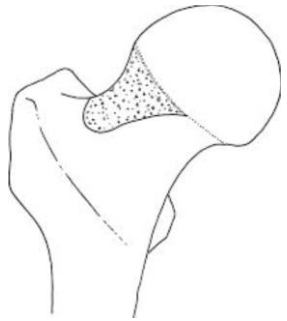
α>β



Muscle

Metabolic Rate

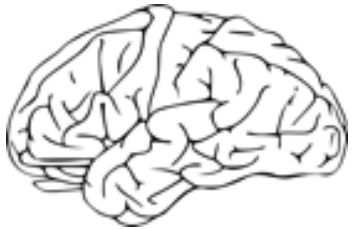
α>β



Bone

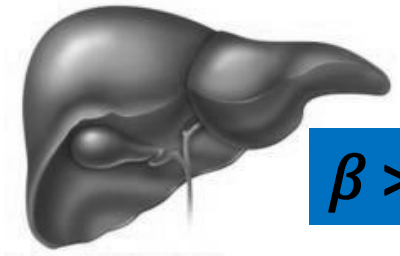
Bone maturation
Bone turnover
Growth

α>β



CNS

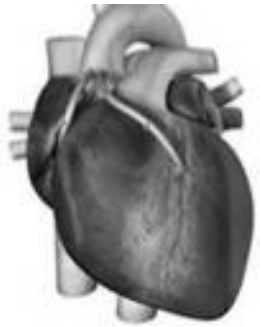
Brain development
Myelination



β >α

Liver

Cholesterol metabolism



α>β

Heart

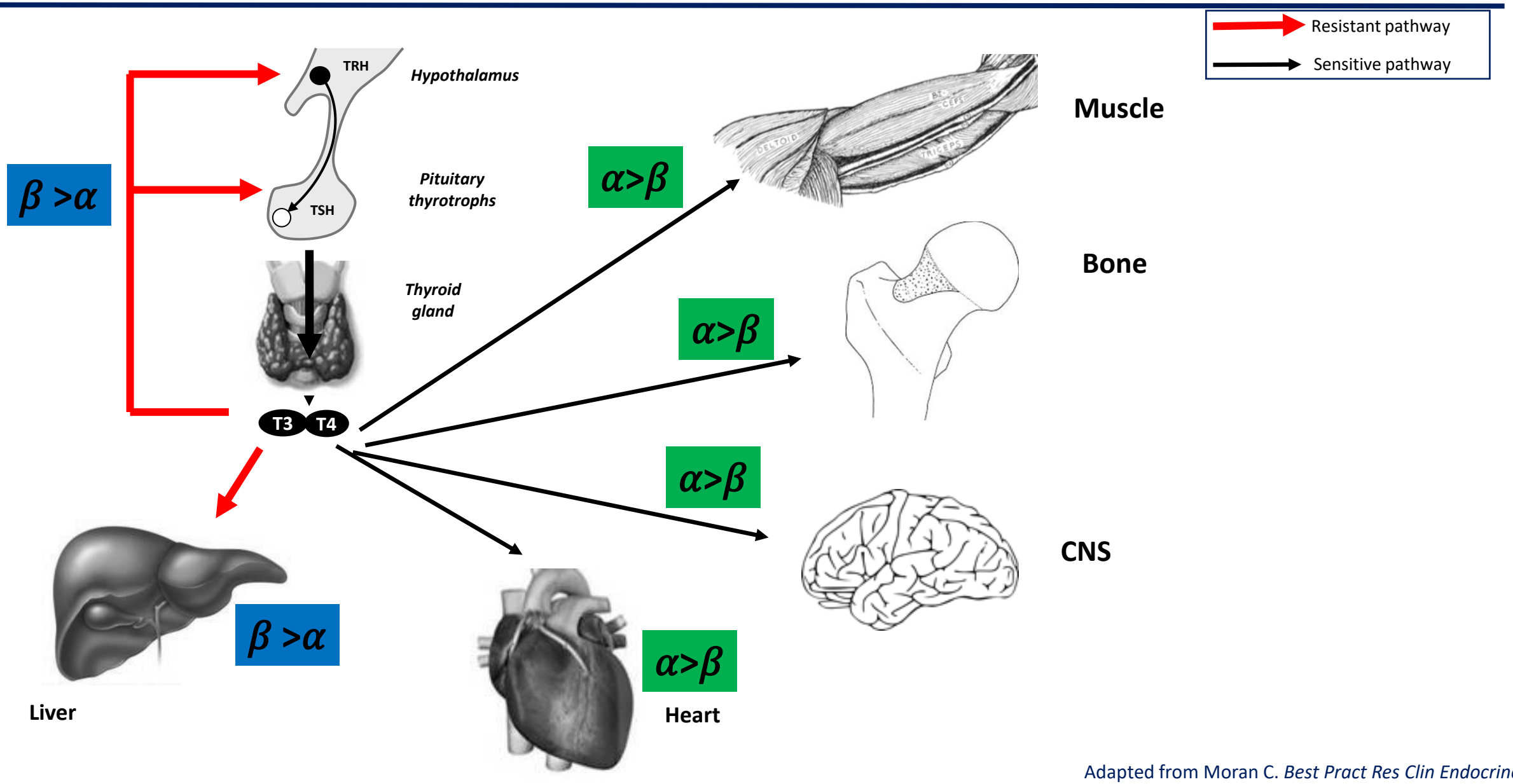
Inotropic effects
Chronotropic effects

Adapted from Moran C. *Best Pract Res Clin Endocrinol Metab.* 2015. PMID 26303090

Overview

2.What is Resistance to Thyroid Hormone β?

RTHβ: Thyroid Receptor β is Resistant to Thyroid Hormone

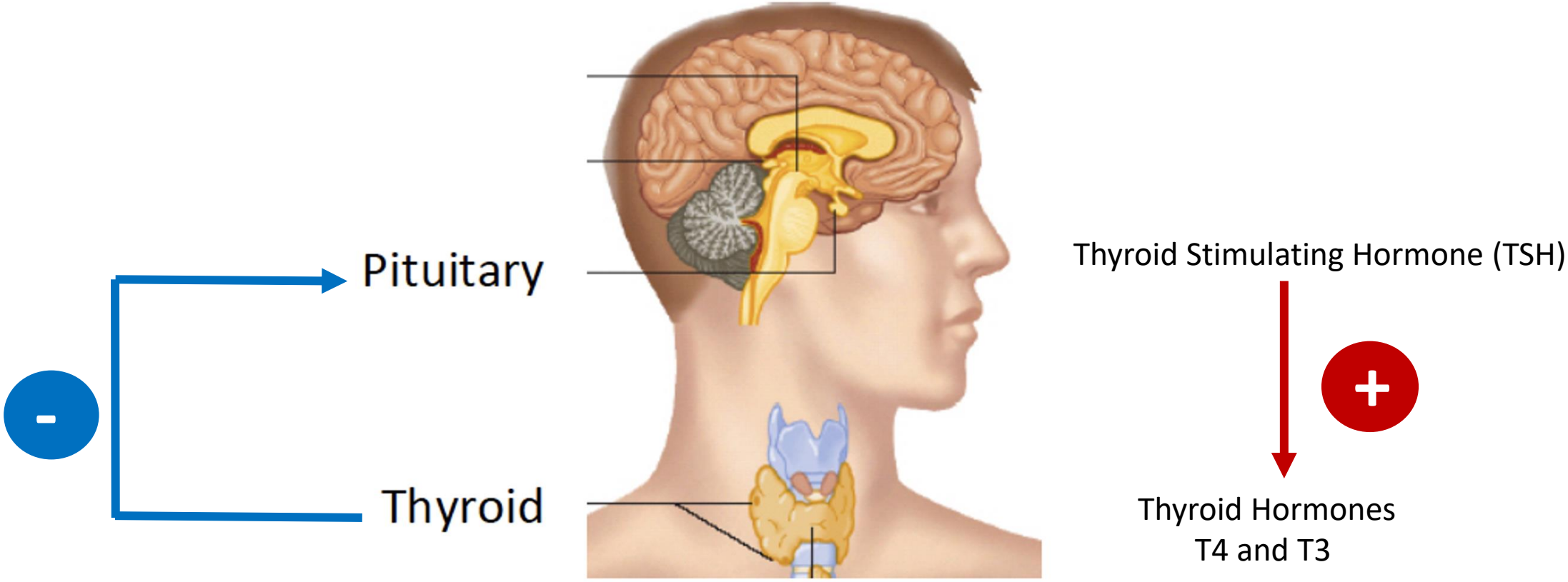


Adapted from Moran C. *Best Pract Res Clin Endocrinol Metab.* 2015. PMID 26303090

Overview

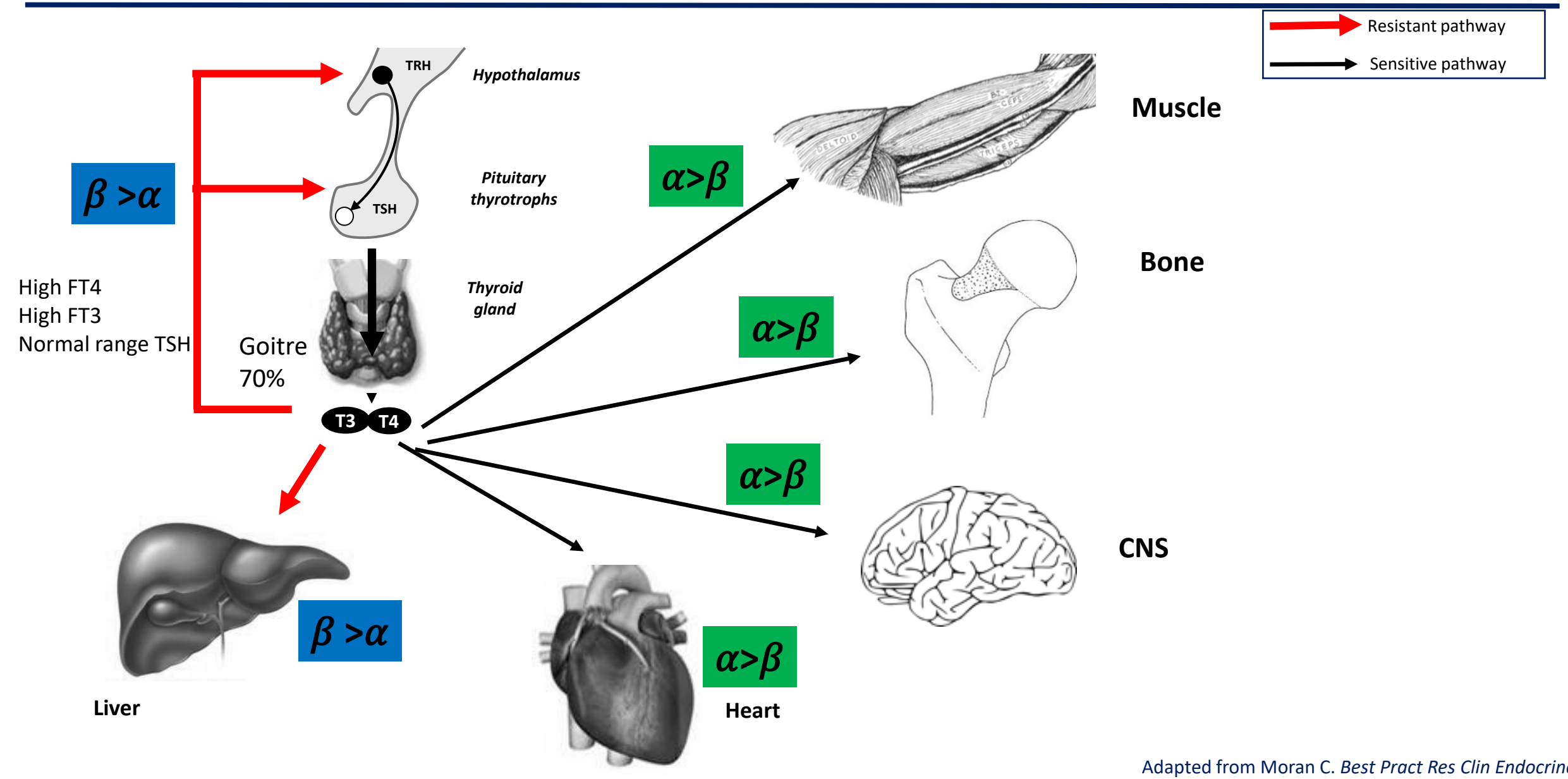
3.How is Resistance to Thyroid Hormone β diagnosed?

“The Feedback Loop” in RTHβ



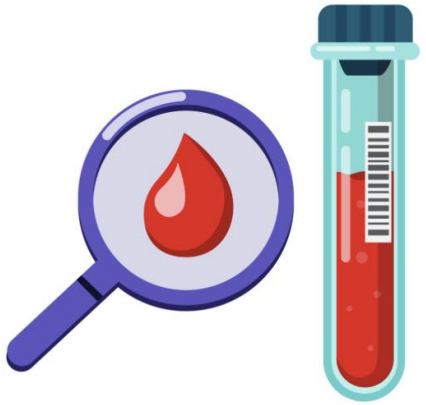
		Example levels	Normal Levels
TSH	NORMAL RANGE	4.0	0.27-4.2
T4	HIGH	45	12-22
T3	HIGH	22	3.1-6.8

RTHβ: Abnormal Thyroid blood tests

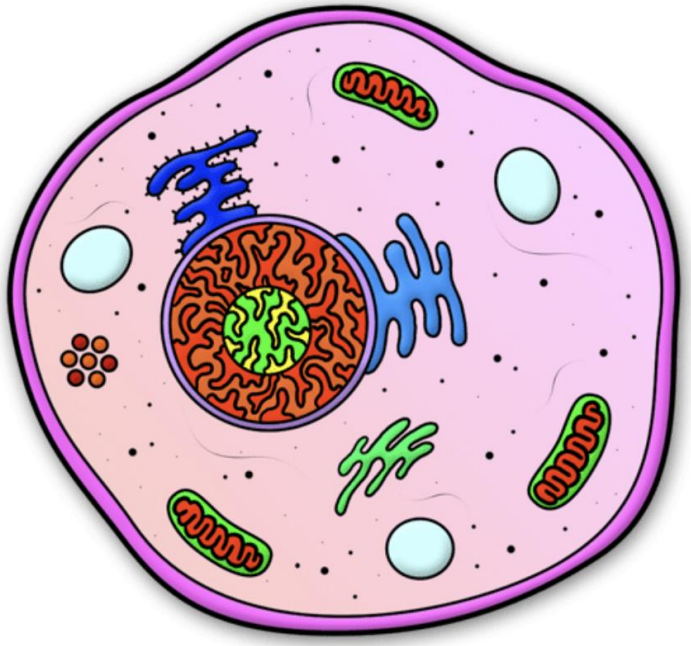


Adapted from Moran C. *Best Pract Res Clin Endocrinol Metab.* 2015. PMID 26303090

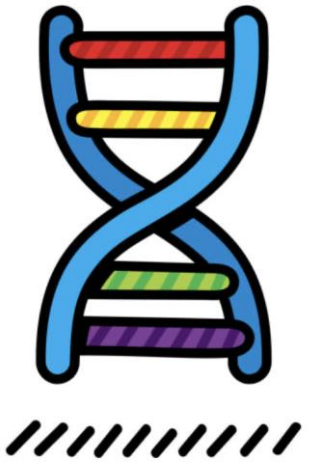
DNA testing for RTHβ



Blood test



Cell



DNA

“THRB”

Gene

Inheritance of RTHβ

Usually inherited in an autosomal dominant pattern

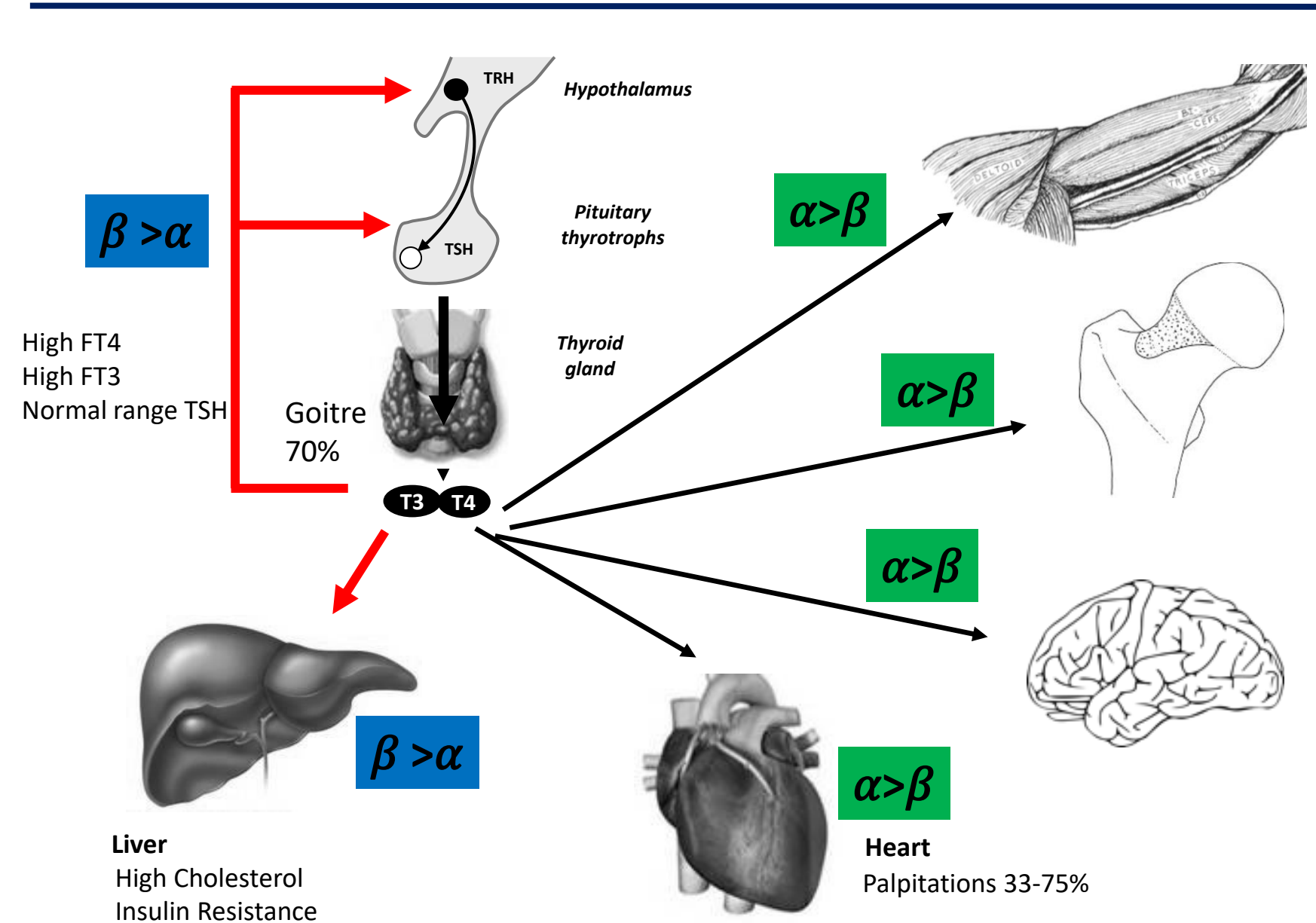
So for a father/mother with RTHβ, each of their children has a 1 in 2 chance of having the condition

1 in 20,000 to 40,000
Males = Females
Can be diagnosed at any age

Overview

4. Effects of Resistance to Thyroid Hormone β

RTHβ: Summary of Features



Muscle
Raised Metabolic Rate
Failure to Thrive in Childhood

Bone
Low BMD
Delayed bone age 29-47%
Short stature 18-25%

Also:
ENT infections 55%
Hearing impairment 10-22%

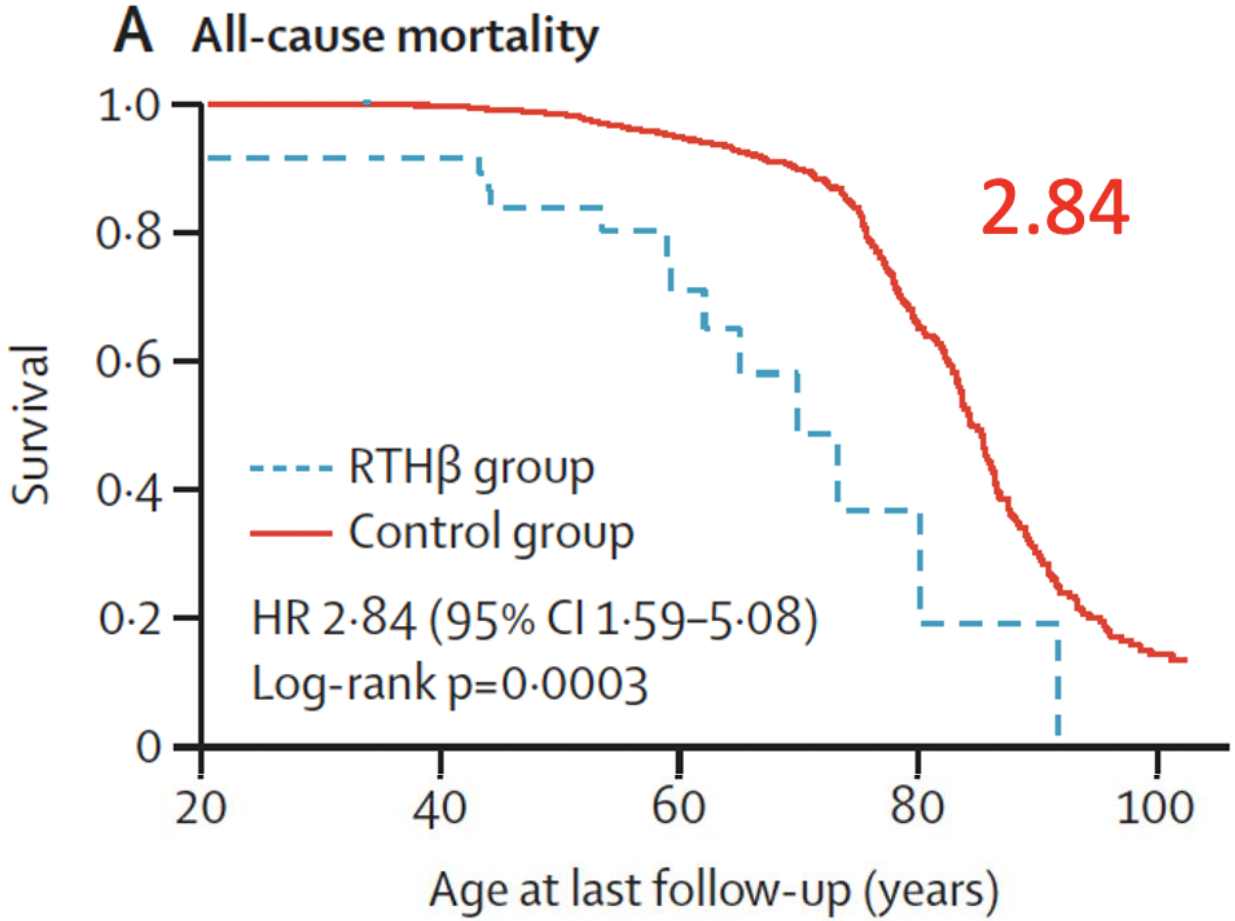
CNS
ADHD 40-60%
Poor attention, concentration
Reduced IQ 30%
Anxiety
Hyperkinetic behaviour 33-68%

Liver
High Cholesterol
Insulin Resistance

Heart
Palpitations 33-75%

Adapted from Moran C. *Best Pract Res Clin Endocrinol Metab.* 2015. PMID 26303090

Increased Mortality RTHβ

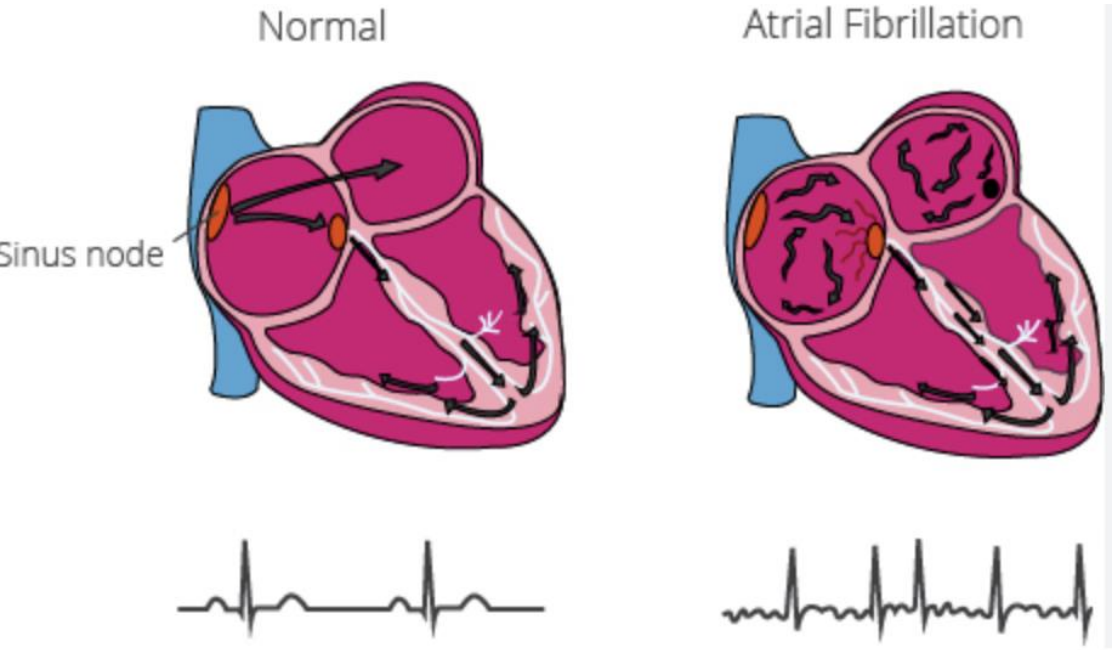


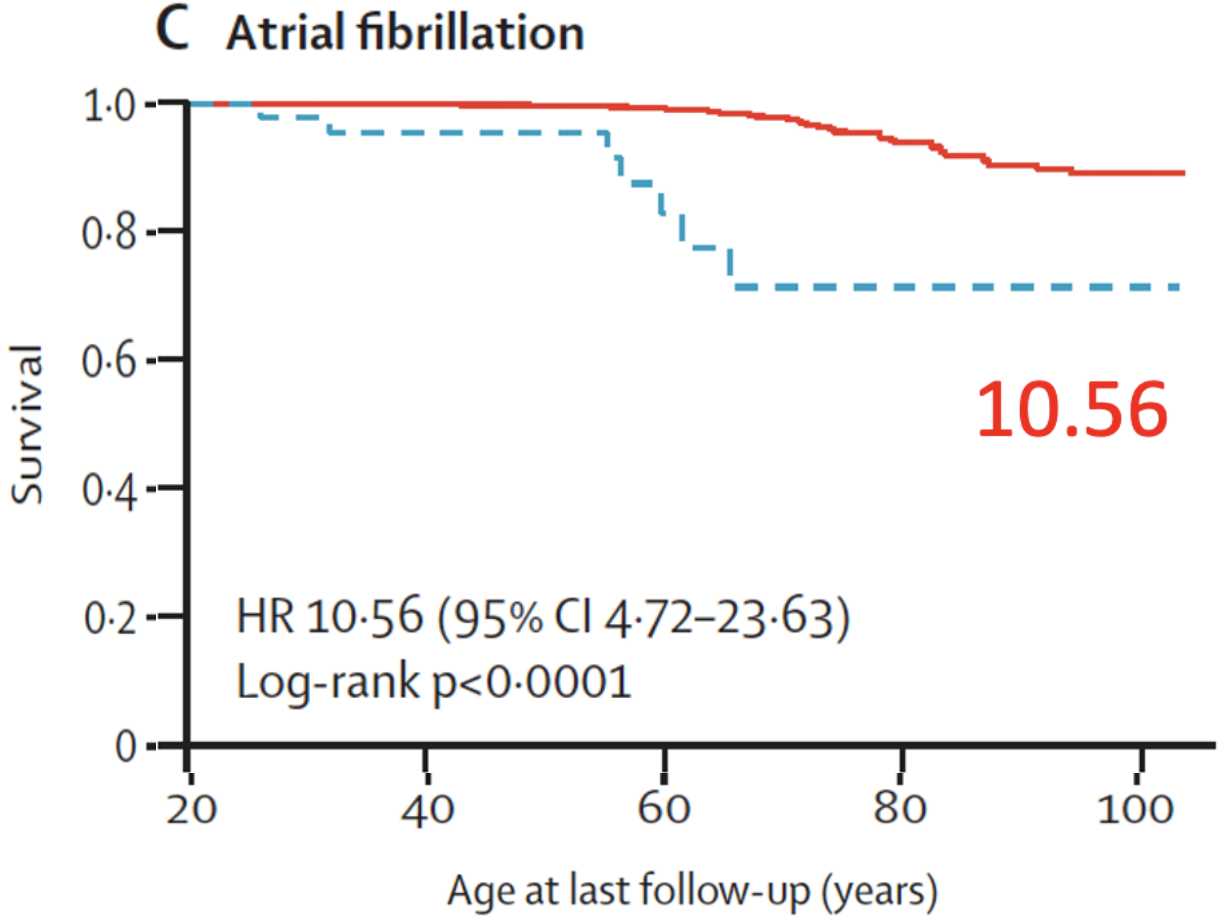
Welsh cohort
55 patients RTH Beta
2750 Age and sex matched controls

Okosieme, Lancet Diabetes Endocrinology 2023. PMID 37475119

Features of RTHβ: focus on heart

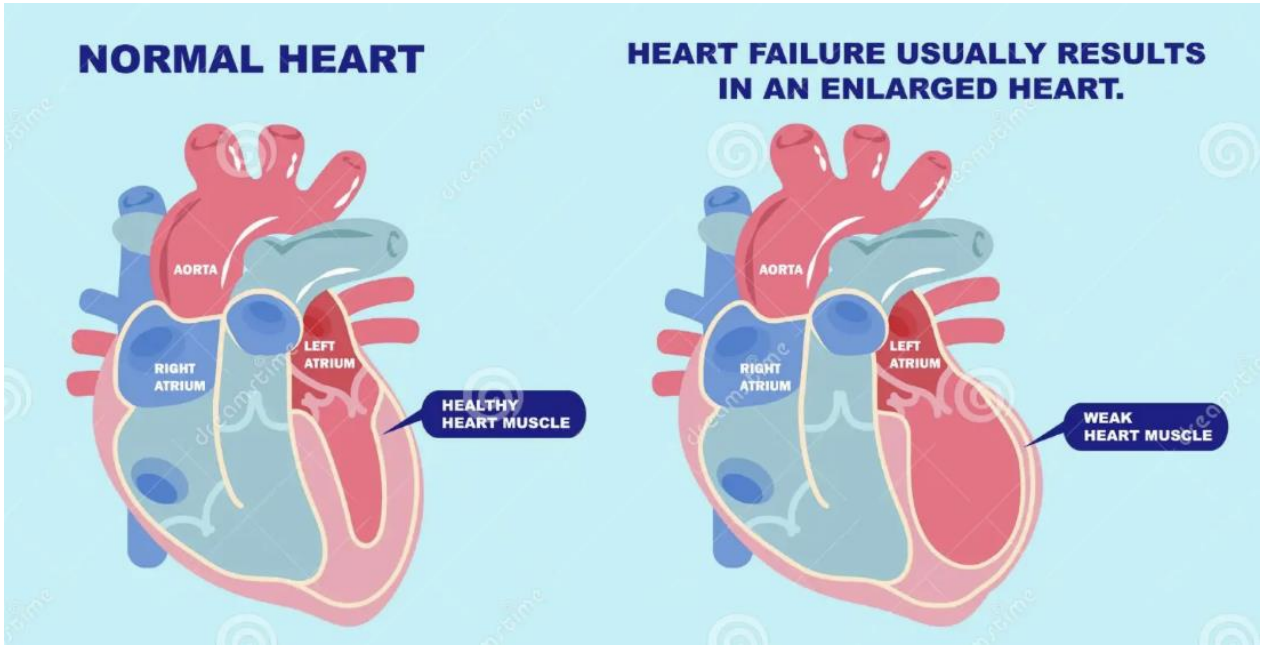
Atrial Fibrillation
(Risk is increased)

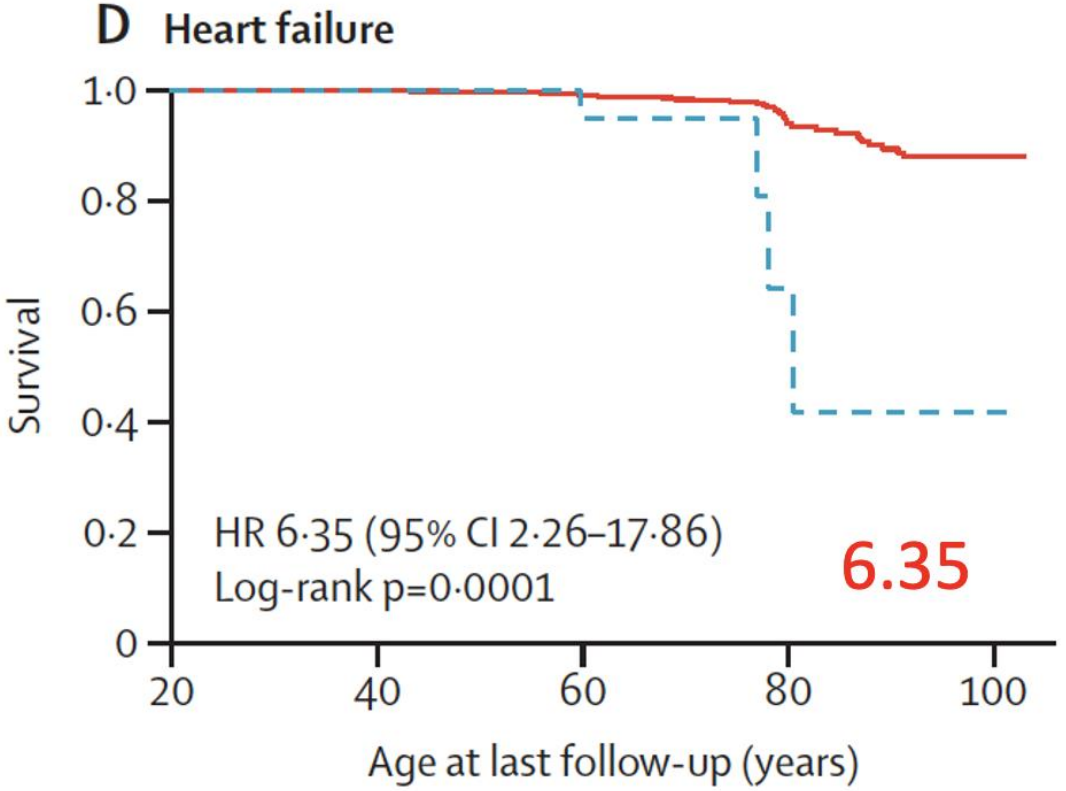




Features of RTHβ: focus on heart

Heart Failure
(Risk is increased)



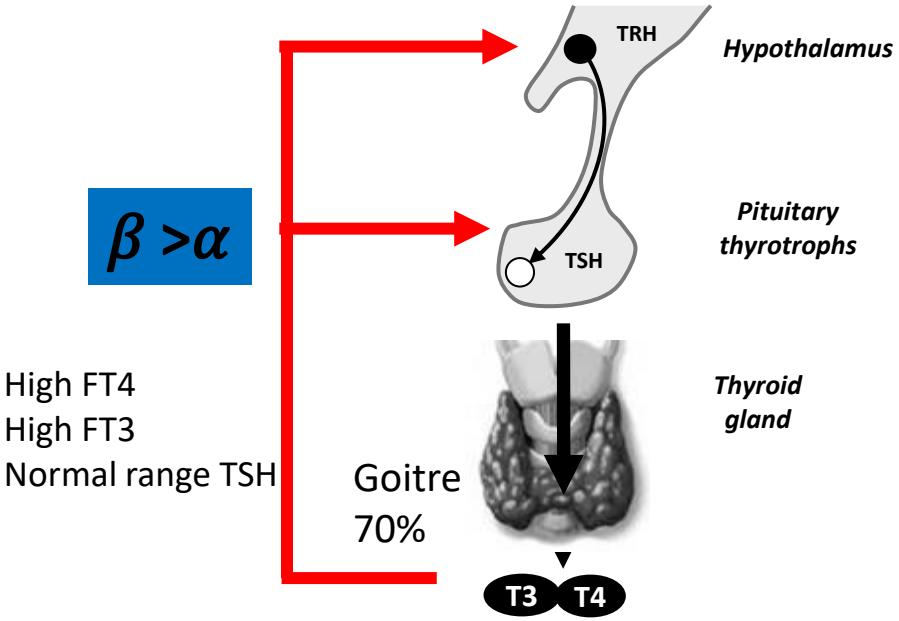


Median age 1st event 56 (RTH) vs 67 years

Overview

4. Treatment options, Unmet Needs

RTHβ: Treatment



Current treatment options are not optimal

Conventional Treatments for an Overactive Thyroid Gland not recommended

- Anti-thyroid drugs
- Surgery to remove Thyroid gland
- Radioiodine treatment

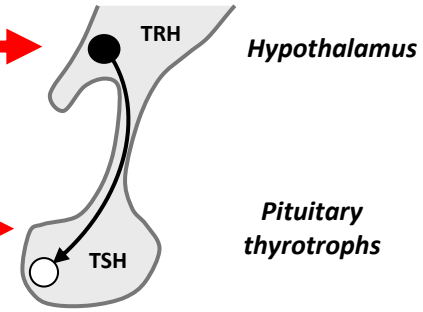
These do not address the imbalance in Thyroid Hormone exposure in all tissues

Adapted from Moran C. *Best Pract Res Clin Endocrinol Metab.* 2015. PMID 26303090

RTHβ: Treatments

Resistant pathway

Sensitive pathway



Hypothalamus

Pituitary thyrotrophs

Thyroid gland

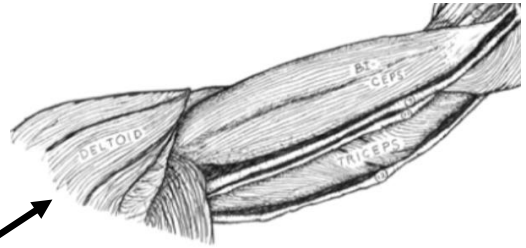
β >α

High FT4
High FT3
Normal range TSH

Goitre 70%

T3 T4

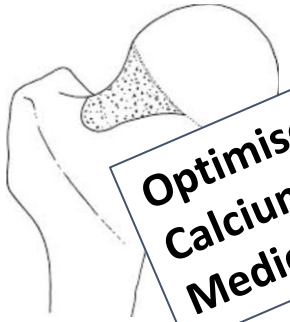
α>β



Muscle

Raised Metabolic Rate
Failure to Thrive in Childhood

α>β

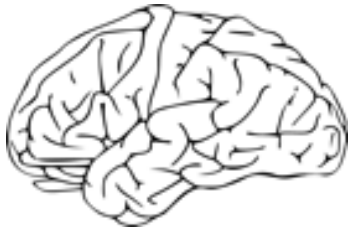


Optimise Bone health
Calcium and Vitamin D
Medication for Bones

ge 29-47%
ture 18-25%

α>β

Also:
ENT infections 55%
Hearing impairment 10-22%



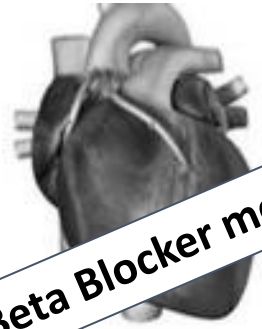
CNS

ADHD 40-60%
Poor attention, concentration
Reduced IQ 30%
Anxiety
Hyperkinetic behaviour 33-68%

β >α

Cholesterol medication

Liver
SHBG
Cholesterol
Insulin Resistance

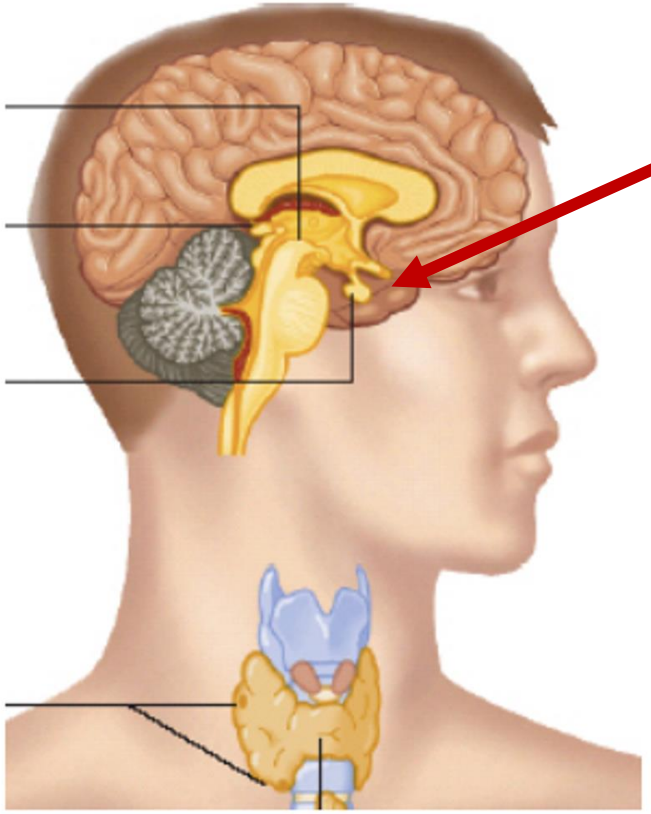


Beta Blocker medication

Heart
Palpitations 33-75%

Adapted from Moran C. *Best Pract Res Clin Endocrinol Metab.* 2015. PMID 26303090

TRIAC Medication



TRIAC

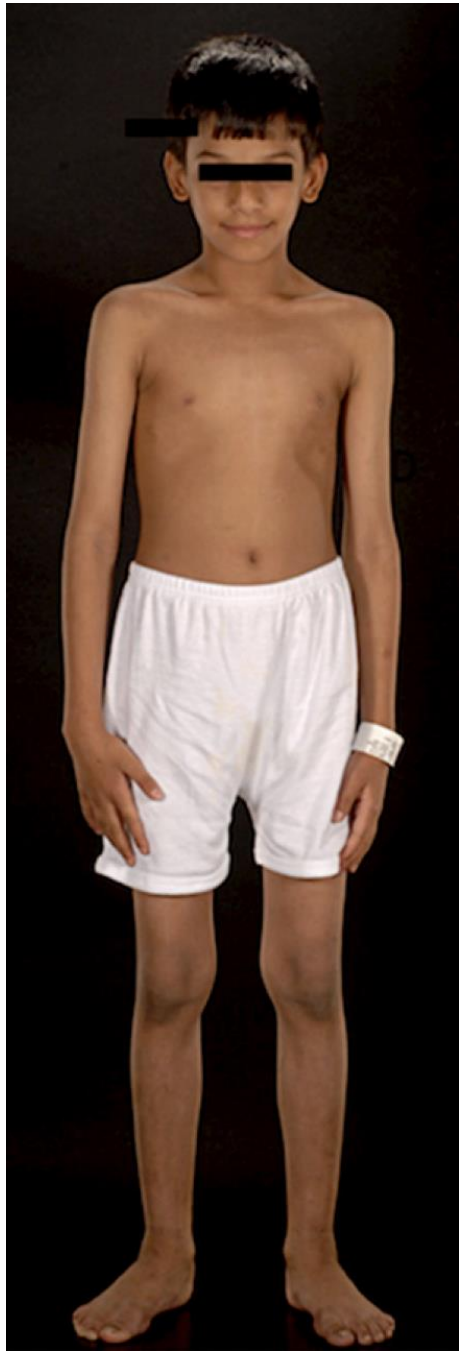
Thyroid Stimulating Hormone (TSH) ↓

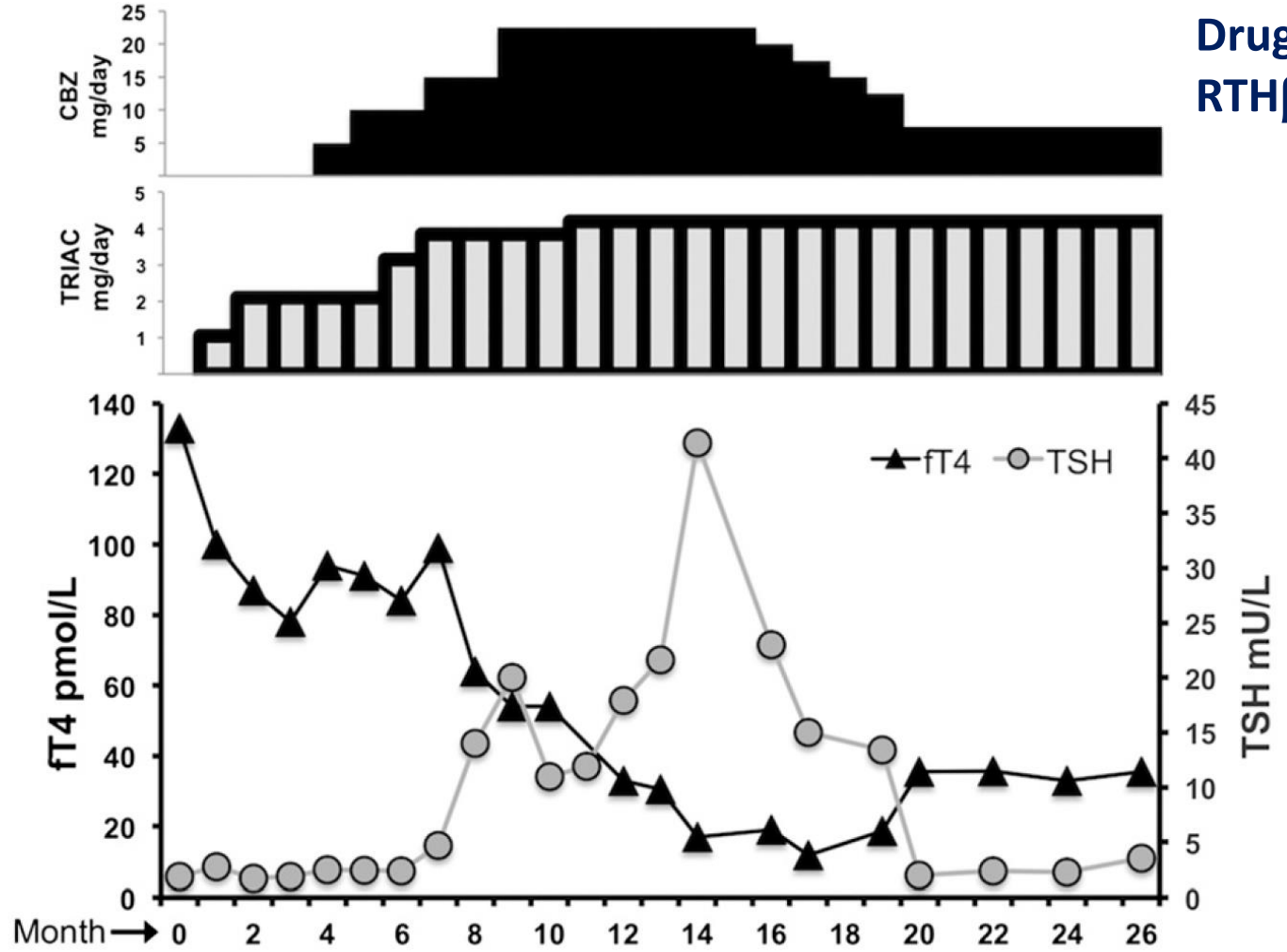
↓

Thyroid Hormones T4 and T3 ↓

TRIAC
= Tri-iodothyroacetic Acid
= Tiratricol
= “Emcitate”

Dual Therapy with Anti-Thyroid Drugs and TRIAC in severe RTHβ



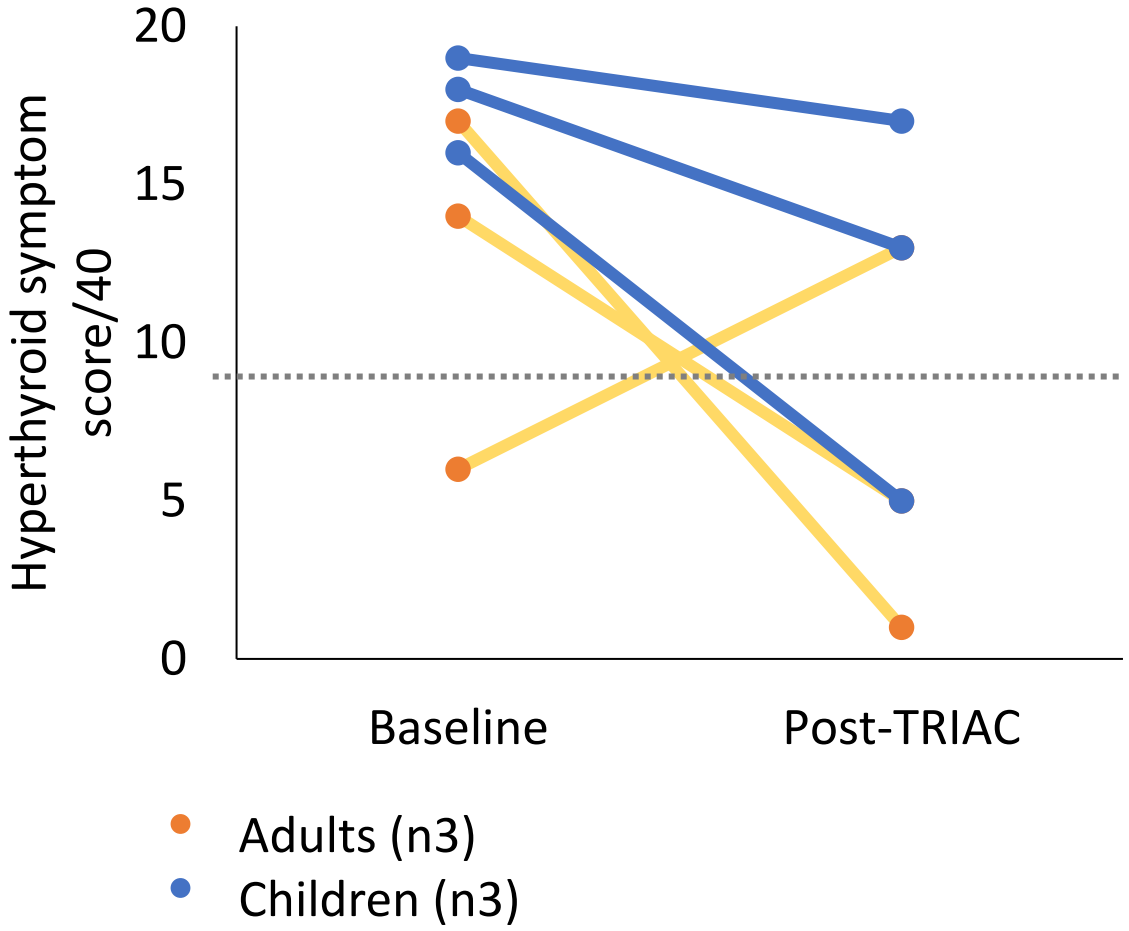


Weight (kg) [LBM (kg)]	20.17 [16.9]	23 [18.3]	23 [19.2]	26.2 [21.03]
REE (kJ/kg LBM)	0.176	0.179	0.161	0.166
Sleeping HR (bpm)	108	83	79	72
NT- ProBNP (pg/ml)	298	91	135	125
LDL (mmol/L)	1.53	1.24	1.46	1.58

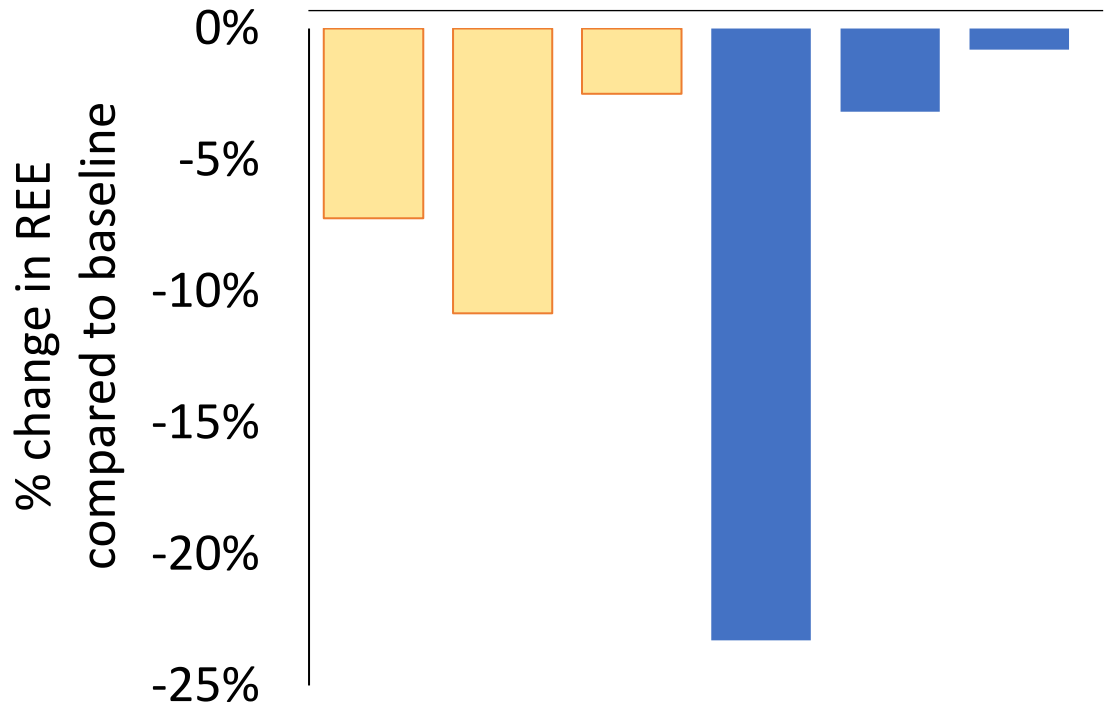
Moran,
J Endocr Soc
2017
PMID 29264576

RTHβ: TRIAC monotherapy

Hyperthyroid symptom score



Change in Metabolic Rate



C Moran VKK Chatterjee, unpublished data

Overview

6. Future Research Priorities

Patient Webinar Feedback

Treatments

Health Registry

Understanding Heart Health

Thank you

Monitoring of RTHβ

Both Children and adults
Children only
Adults only

	How?	How often?
Clinical	Clinical assessment; symptoms Examination (weight, Blood Pressure, goitre, heart exam) Growth, school performance, hearing, behaviour	Annual
Blood tests	Fasting bloods for Cholesterol and Diabetes tests TSH, FT4, FT3	Annual
Scans	DXA scan for bone health Bone age Xray (to assess bone maturation) Ultrasound thyroid scan (sometimes)	Every 2-5 years Every 1-3 years As indicated
Heart Health	ECG (Sticker Test on chest, takes a few minutes) Holter (24 hr monitoring of heart rate) Echocardiogram (ultrasound test of heart)	Annual Every 1-2 years Every 2-3 years
Others	Hearing Test ADHD testing Cardiology consultation request Offer first degree relative screening	If indicated If indicated If indicated If desired

Increased Mortality RTHβ

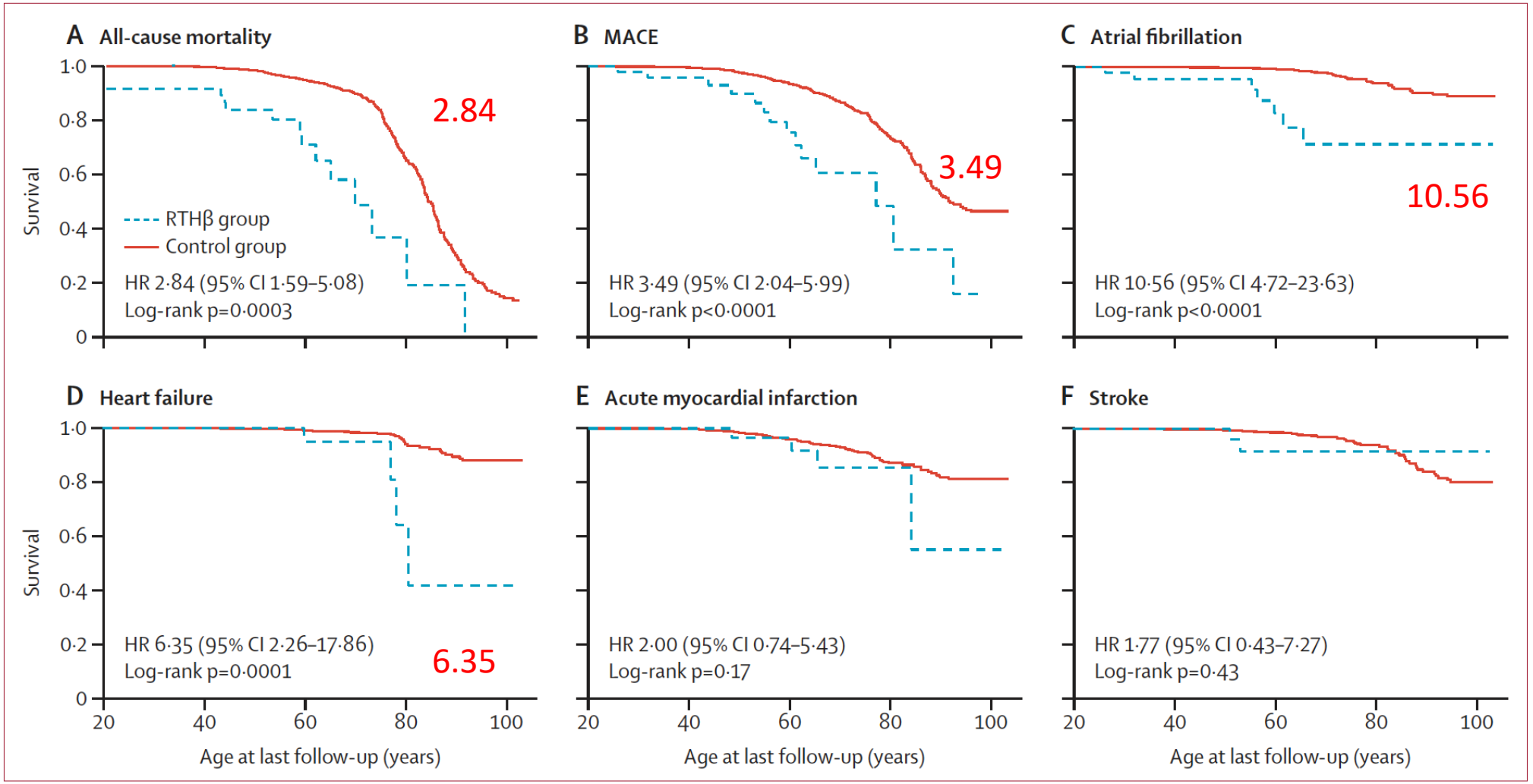


Figure 2: **Kaplan-Meier curves for mortality and cardiovascular events in patients with RTHβ.**

Welsh cohort
55 patients RTH Beta
2750 Age and sex matched controls
Median age 1st event 56 vs 67

Okosieme, Lancet Diabetes Endocrinology 2023. PMID 37475119

WE CARE

FOR THE RARE

Egetis Investor Day 2023

December 19, 2023

Q&A

Dr Carla Moran & Nicklas Westerholm

EGETIS THERAPEUTICS

WE CARE

FOR THE RARE

Concluding remarks

December 19, 2023

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

Building a sustainable orphan drug company

- Successfully develop *Emcitate* for EU & US approvals in 2024/25 and *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

GOALS

WE CARE FOR THE RARE

VISION

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

MISSION

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

Egetis has recently delivered transformative milestones

Jul 24

First patient recruited in ReTRIACt study, which is pivotal for US NDA

Oct 9

Submitted application for regulatory approval of *Emcitate* for MCT8 deficiency in EU (MAA)

Oct 10

Secured long-term financing of SEK 462m, adding top-tier US specialist investor as largest shareholder

- SEK 172m equity private placement (SEK 155m subscribed by Frazier Life Sciences)
- SEK 290m debt financing (BlackRock; EUR 10m & EUR 15m tranches)

Nov 10

License agreement with Fujimoto for *Emcitate* in Japan

Nov 30

EUR 10m draw down of debt facility

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 and consistent data in clinical trials, demonstrating significant effects on key clinical outcomes
  - Supported by strong mechanistical rationale and data from animal models
- High likelihood to reach first approval in 2024, already passed most of typical drug development risks
  - Submitted application for regulatory approval of Emcitate for MCT8 deficiency in EU (MAA) – Submitted October 9, 2023
  - A small and short trial reconfirming the effect on biomarker T3 under way to complete the US dossier
- Significant market opportunity with potential for premium orphan drug pricing
  - Disease awareness activities already bearing fruits
  - Continuous expansion of the Emcitate Managed Access Program confirms high unmet medical need
- Eligible for priority review voucher upon US approval, which can be sold for ~100 MUSD

Under the terms of the acquisition of RTT in Q4 2020, 50% of the potential net proceeds from a sale of a PRV related to Emcitate should be paid as an earnout to the sellers of RTT

EGETIS THERAPEUTICS

WE CARE

FOR THE RARE

Egetis Investor Day 2023

December 19, 2023

Thank You