EGETIS THERAPEUTICS

WE CARE

FOR THE RARE

Investor Day

December 18, 2024

Nicklas Westerholm, CEO

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Tiratricol (Emcitate®) is under development for the treatment of patients with MCT8 deficiency and is not EMA/FDA-approved. Safety and efficacy have not been established.

PRESS

Egetis receives positive CHMP opinion for Emcitate® (tiratricol) for the treatment of MCT8 deficiency

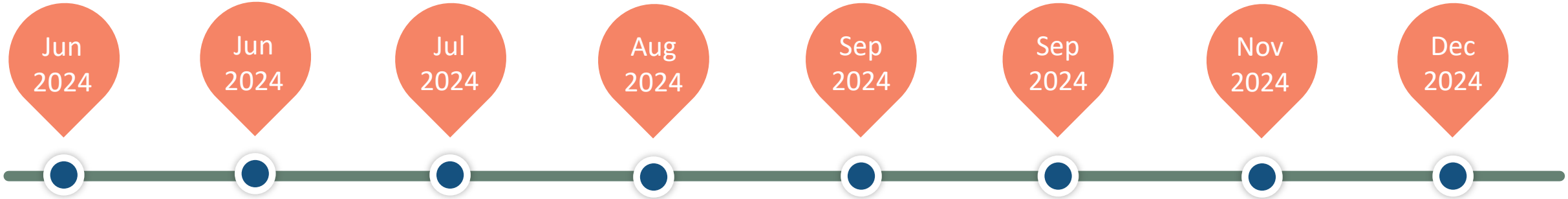
December 12, 2024

Stockholm, Sweden, December 12, 2024. Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion for Emcitate® (tiratricol) for "The treatment of MCT8 deficiency". The European Commission, which grants central marketing authorizations in the European Union (EU), will review the CHMP recommendation and is expected to make a final decision within 67 days. If approved, tiratricol will become the first approved drug which addresses MCT8 deficiency.

Agenda: Egetis Investor Day, December 18, 2024

Time (CET/ET)	Subject	Presenter(s)
15:00/9.00am	Welcome & corporate update	Nicklas Westerholm, CEO
15:10/9.10am	MCT8 deficiency: recent advances with tiratricol	Prof. Edward Visser, Erasmus Medical Center, NL
15:35/9.35am	Q&A	Visser & Westerholm
15:45/9.45am	Global launch preparations	Henrik Krook, Raymond Francot, Henna Oittinen-Corbinelli, Peter Verwaijen
16:20/10.20am	Q&A	Krook, Francot, Oittinen-Corbinelli, Verwaijen, Westerholm
16:30/10.30am	Break	
16:50/10.50am	US regulatory pathway & ReTRIACt study	Westerholm
17:00/11.00am	US opportunity for *Emcitate*	Anny Bedard, Ann-Marie Redmond
17:15/11.15am	Q&A	Bedard, Redmond, Westerholm
17:25/11.25am	RTH-beta and the unmet medical need	Prof. Aled Rees, Cardiff University, UK
17:50/11.50am	Q&A	Rees & Westerholm
17:55/11.55am	Concluding remarks	Mats Blom, Chairman of the Board
18:00/12.00pm	Ends	

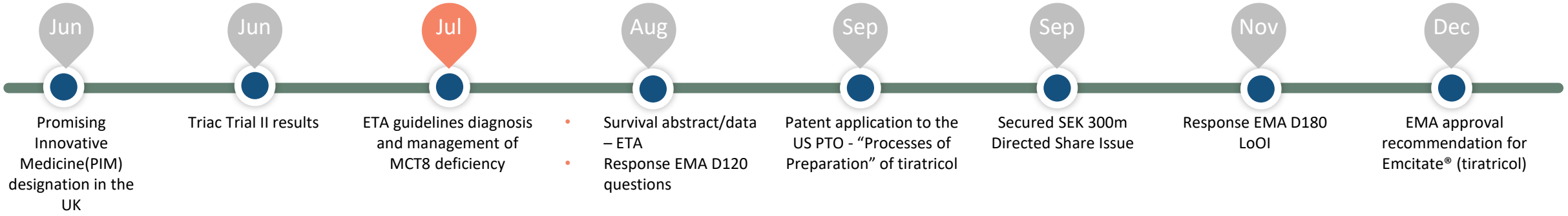
Several important milestones over the last 6 months



- Promising Innovative Medicine(PIM) designation in the UK
- Triac Trial II results
- ETA guidelines diagnosis and management of MCT8 deficiency
- Survival abstract/data – ETA
- Response EMA D120 questions
- Patent application to the US PTO - “Processes of Preparation” of tiratricol
- Secured SEK 300m Directed Share Issue
- Response EMA D180 LoOI
- EMA approval recommendation for Emcitate® (tiratricol)

ETA: European Thyroid Association

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



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

PRESS

European Thyroid Association recommends tiratricol (Emcitate®) as long-term therapy for all patients with MCT8 deficiency in new guidelines

July 17, 2024

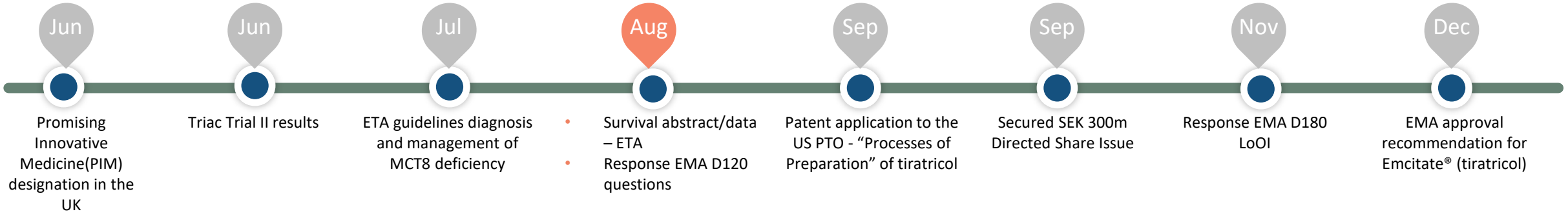
Stockholm, Sweden, July 17, 2024. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (Nasdaq Stockholm: EGTX), today announced that the European Thyroid Association (ETA) has published new guidelines recommending the use of tiratricol (TRIAC or Emcitate®) as long-term therapy for all patients with MCT8 deficiency, and for certain patients with Resistance to Thyroid Hormone (RTH)-beta, as further outlined in the guidelines.

There are currently no approved treatments for MCT8 deficiency or RTH-beta. Egetis has obtained orphan drug designation for tiratricol for the treatment of MCT8 deficiency and RTH-beta in the EU and the USA, and has submitted a marketing authorisation application in the EU, which is currently under review by the European Medicines Agency.

These inaugural *2024 European Thyroid Association Guidelines on diagnosis and management of genetic disorders of thyroid hormone transport, metabolism and action* were commissioned by the Executive Committee of the ETA and developed by an independent team of experts. The guidelines can be accessed here:

https://etj.bioscientifica.com/view/journals/etj/aop/etj-24-0125/etj-24-0125.xml

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



- Abstract published ahead of the ETA Annual Meeting reports that treatment with tiratricol (Emcitate®) in patients with MCT8 deficiency is associated with a 3x lower risk of mortality.
- Retrospective real-world cohort study investigated the effects of tiratricol on mortality in 228 patients with MCT8 deficiency.
- Tiratricol-treated patients had an approximately three times lower risk of all-cause mortality (Hazard Ratio= 0.28, 95% Confidence Interval= 0.09–0.91, p-value <0.05).

PRESS

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

August 21, 2024

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

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

Egetis submits patent application to the USPTO

Jun — Promising Innovative Medicine(PIM) designation in the UK

Jun — Triac Trial II results

Jul — ETA guidelines diagnosis and management of MCT8 deficiency

Aug
- Survival abstract/data – ETA
- Response EMA D120 questions

Sep — Patent application to the US PTO - "Processes of Preparation" of tiratricol

Sep — Secured SEK 300m Directed Share Issue

Nov — Response EMA D180 LoOI

Dec — EMA approval recommendation for Emcitate® (tiratricol)

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

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

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

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

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

Jun — Promising Innovative Medicine(PIM) designation in the UK

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- Led by Frazier Life Sciences with a USD 10 million investment.
- The Directed Issue was oversubscribed and included both existing and new international and Swedish institutional investors.
- Subscription price at market price.

PRESS

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

September 30, 2024

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

Egetis receives positive CHMP opinion

Jun — Promising Innovative Medicine(PIM) designation in the UK

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

PRESS

Egetis receives positive CHMP opinion for Emcitate® (tiratricol) for the treatment of MCT8 deficiency

December 12, 2024

Stockholm, Sweden, December 12, 2024. Egetis Therapeutics AB (publ) (Nasdaq Stockholm: EGTX) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion for Emcitate® (tiratricol) for "The treatment of MCT8 deficiency". The European Commission, which grants central marketing authorizations in the European Union (EU), will review the CHMP recommendation and is expected to make a final decision within 67 days. If approved, tiratricol will become the first approved drug which addresses MCT8 deficiency.

Building a sustainable orphan drug company

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

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

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

Key upcoming milestones 2025-2026

Emcitate® — **2025-2026**

MCT8 deficiency

- EU approval and launch
- Topline results ReTRIACt for US NDA
- Filing US NDA – priority review
- Middle East & North Africa partnership/s
- Japan – Development plan agreed with PMDA
- US Patent granted - Processes and compounds
- US approval and launch
- US Rare Pediatric Disease Priority Review Voucher

RTH-beta

- Potential initiation of Investigator Initiated Study - Egetis Industry collaborator

Agenda: Egetis Investor Day, December 18, 2024

Time (CET/ET)	Subject	Presenter(s)
15:00/9.00am	Welcome & corporate update	Nicklas Westerholm, CEO
15:10/9.10am	MCT8 deficiency: recent advances with tiratricol	Prof. Edward Visser, Erasmus Medical Center, NL
15:35/9.35am	Q&A	Visser & Westerholm
15:45/9.45am	Global launch preparations	Henrik Krook, Raymond Francot, Henna Oittinen-Corbinelli, Peter Verwaijen
16:20/10.20am	Q&A	Krook, Francot, Oittinen-Corbinelli, Verwaijen, Westerholm
16:30/10.30am	Break	
16:50/10.50am	US regulatory pathway & ReTRIACt study	Westerholm
17:00/11.00am	US opportunity for *Emcitate*	Anny Bedard, Ann-Marie Redmond
17:15/11.15am	Q&A	Bedard, Redmond, Westerholm
17:25/11.25am	RTH-beta and the unmet medical need	Prof. Aled Rees, Cardiff University, UK
17:50/11.50am	Q&A	Rees & Westerholm
17:55/11.55am	Concluding remarks	Mats Blom, Chairman of the Board
18:00/12.00pm	Ends	

MCT8 deficiency: recent advances with tiratricol (Triac)

Edward Visser

Erasmus MC, Rotterdam, The Netherlands

Erasmus MC
University Medical Center Rotterdam

Disclosure

Erasmus MC receives royalties and service fees from Egetis Therapeutics (no personal benefits)

Outline

Brief context of thyroid hormone signaling

MCT8 deficiency: key features & mechanisms of disease

Triac Trial I

Real world data

QoL, survival data & Triac Trial II

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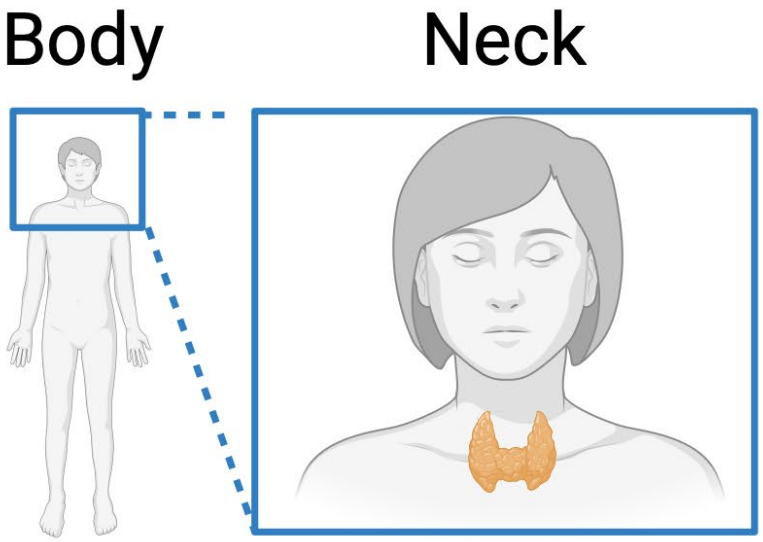
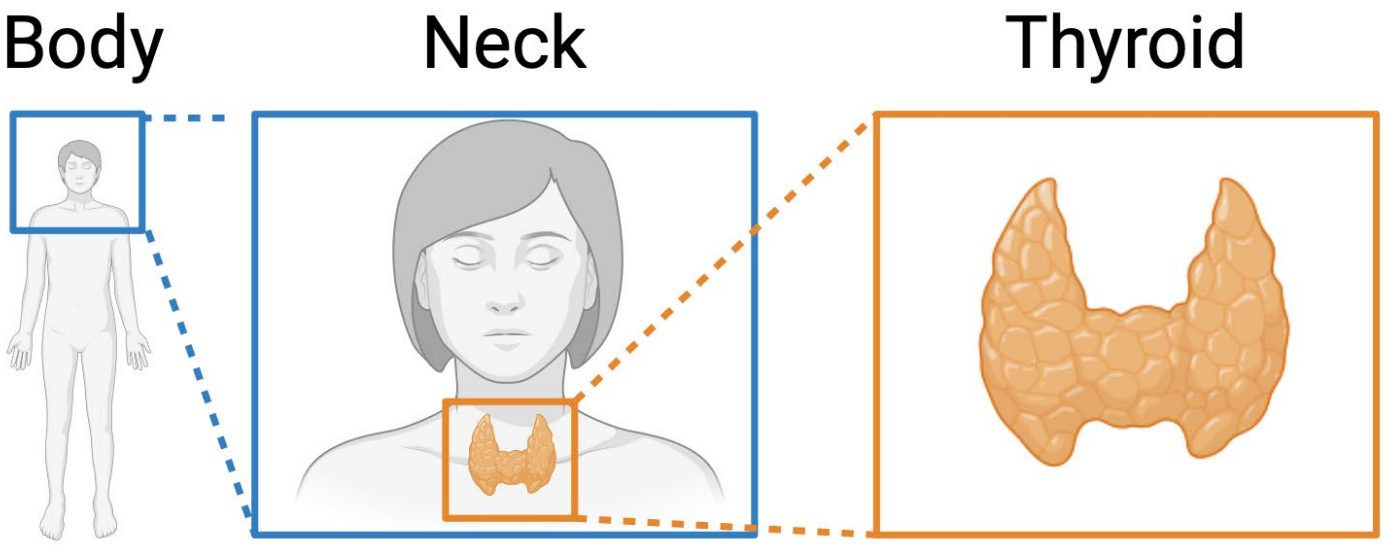
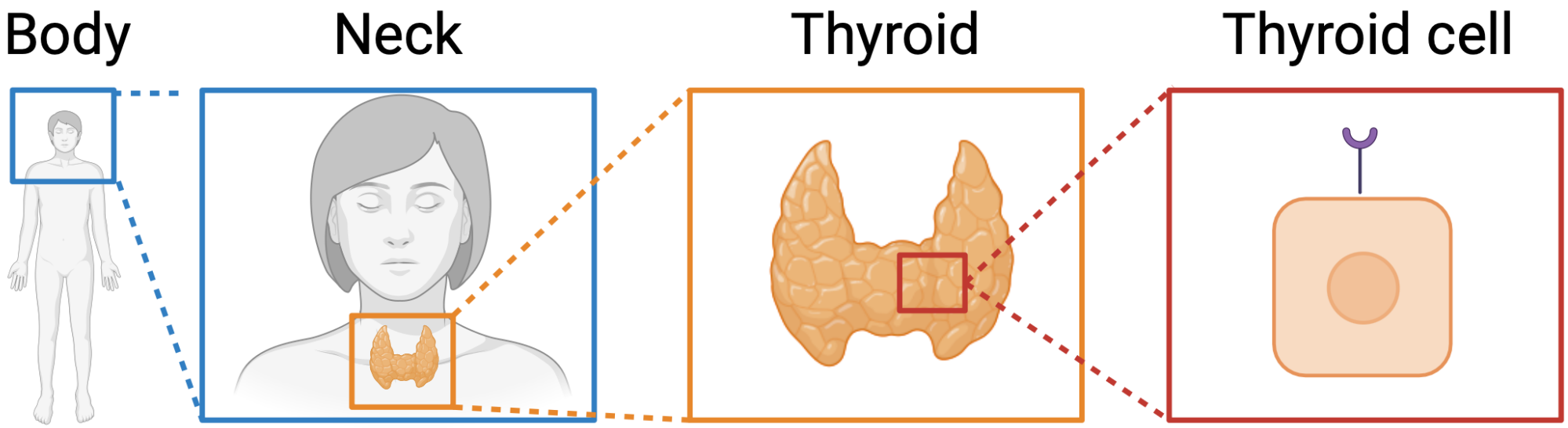
Thyroid gland produces thyroid hormones



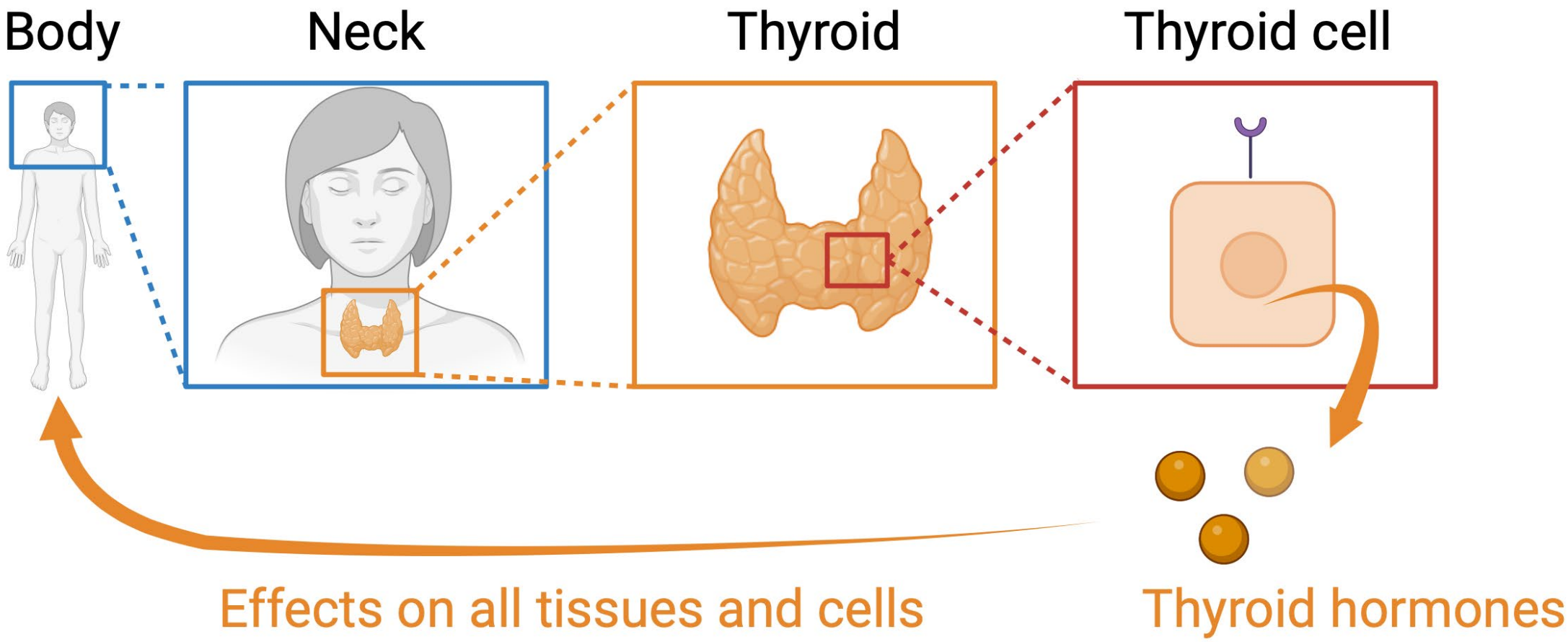
Thyroid gland produces thyroid hormones



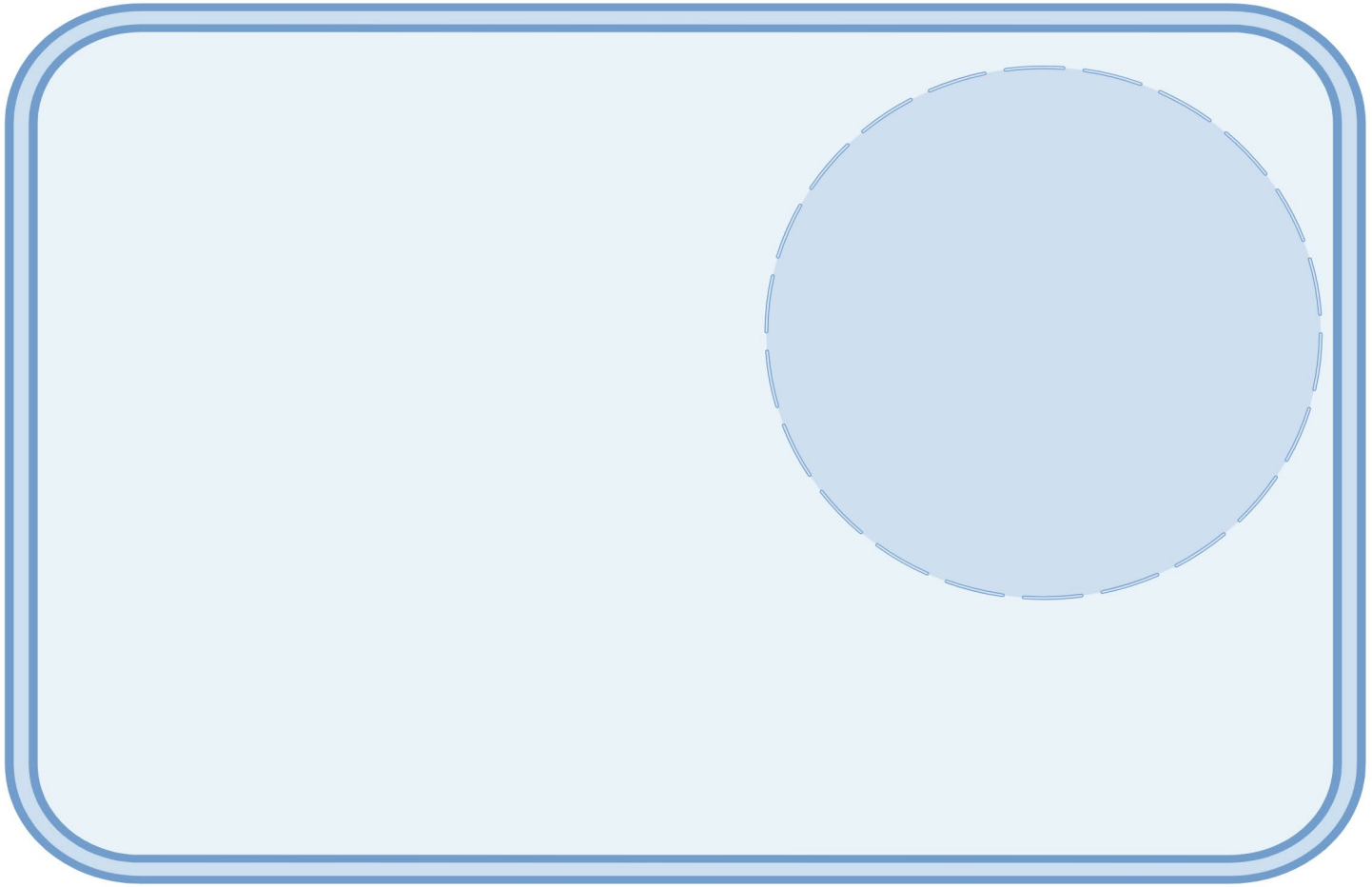
Thyroid gland produces thyroid hormones



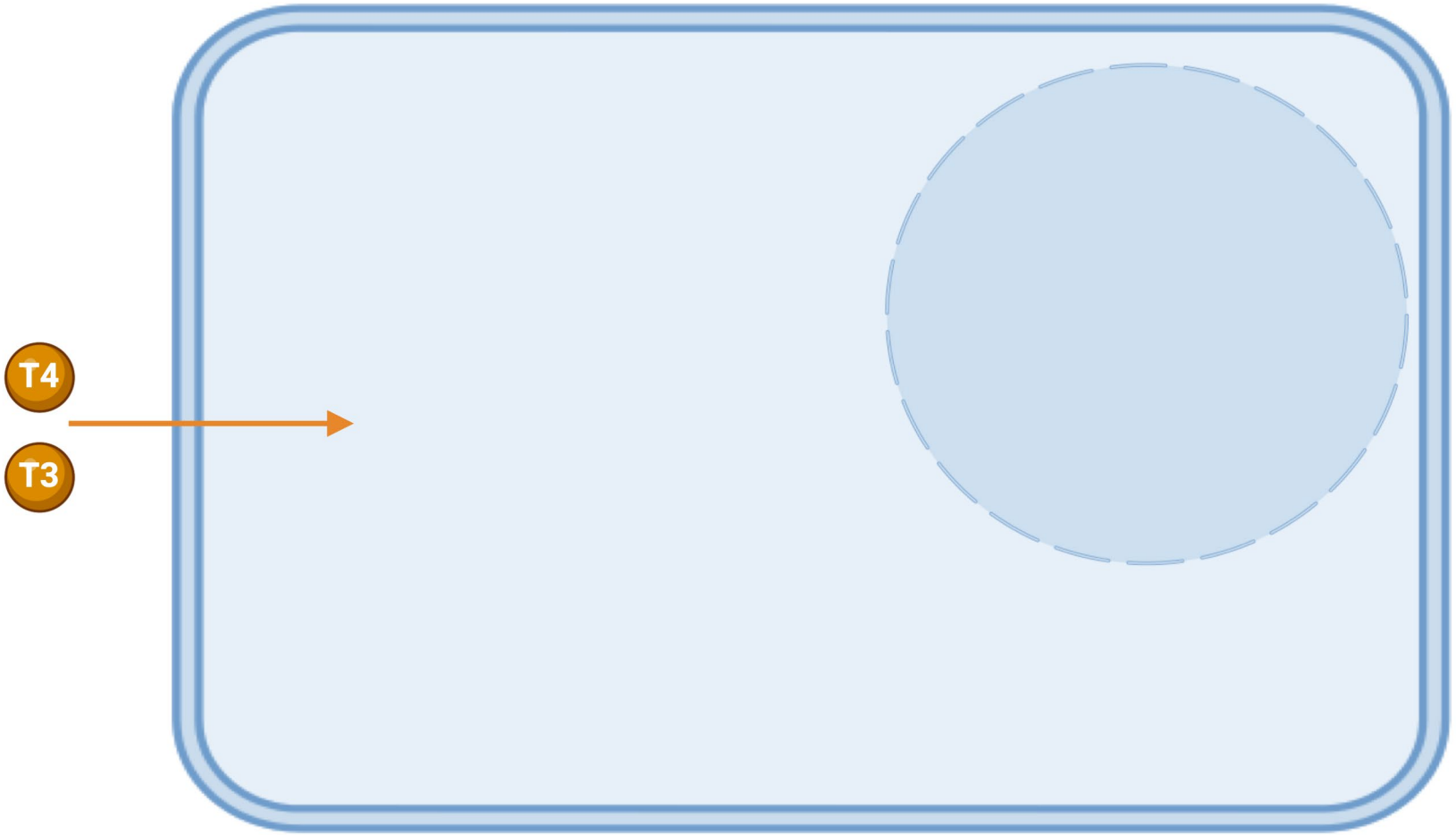
Thyroid gland produces thyroid hormones



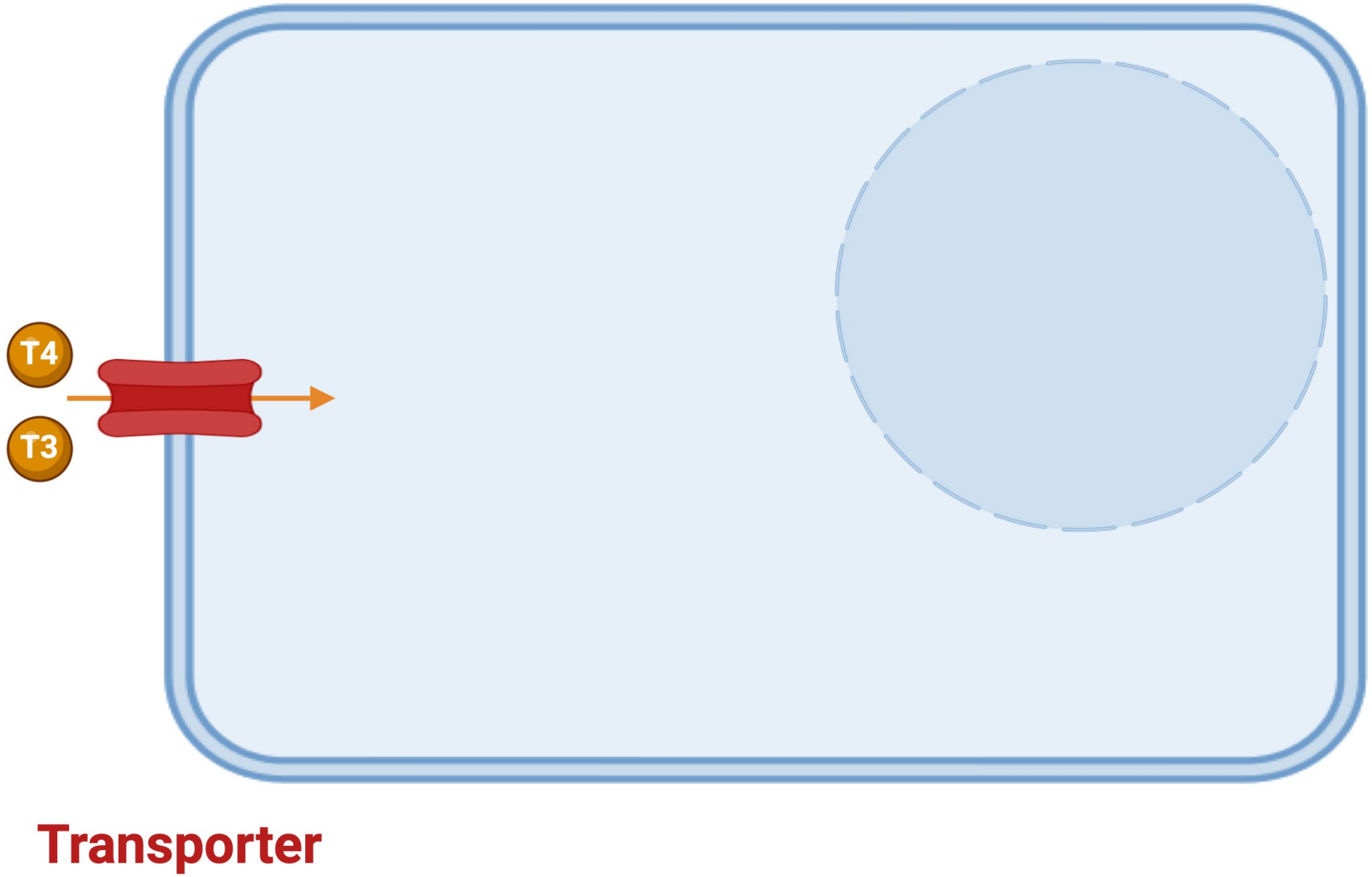
T3 target cell



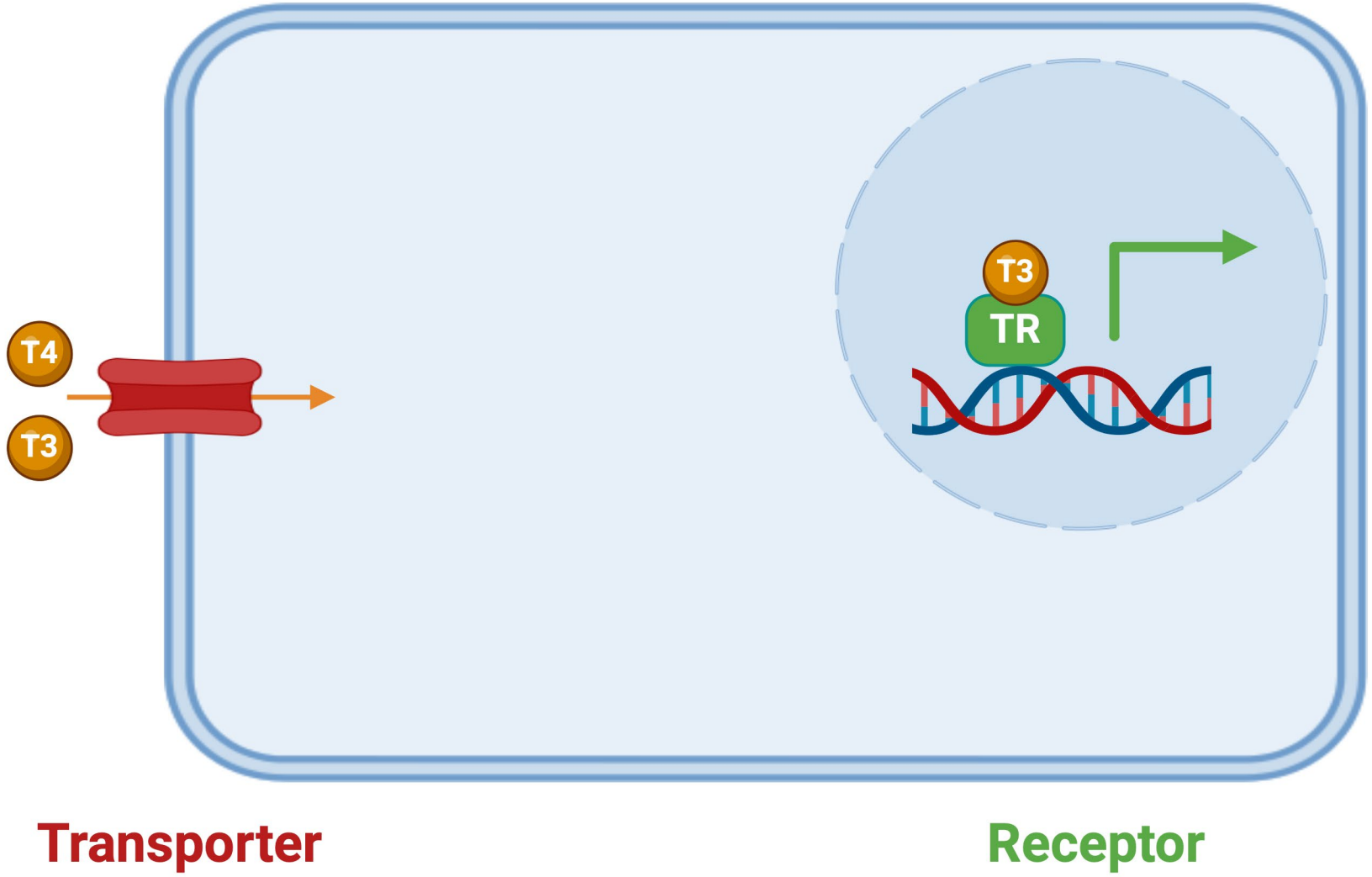
T3 target cell: hormones enter



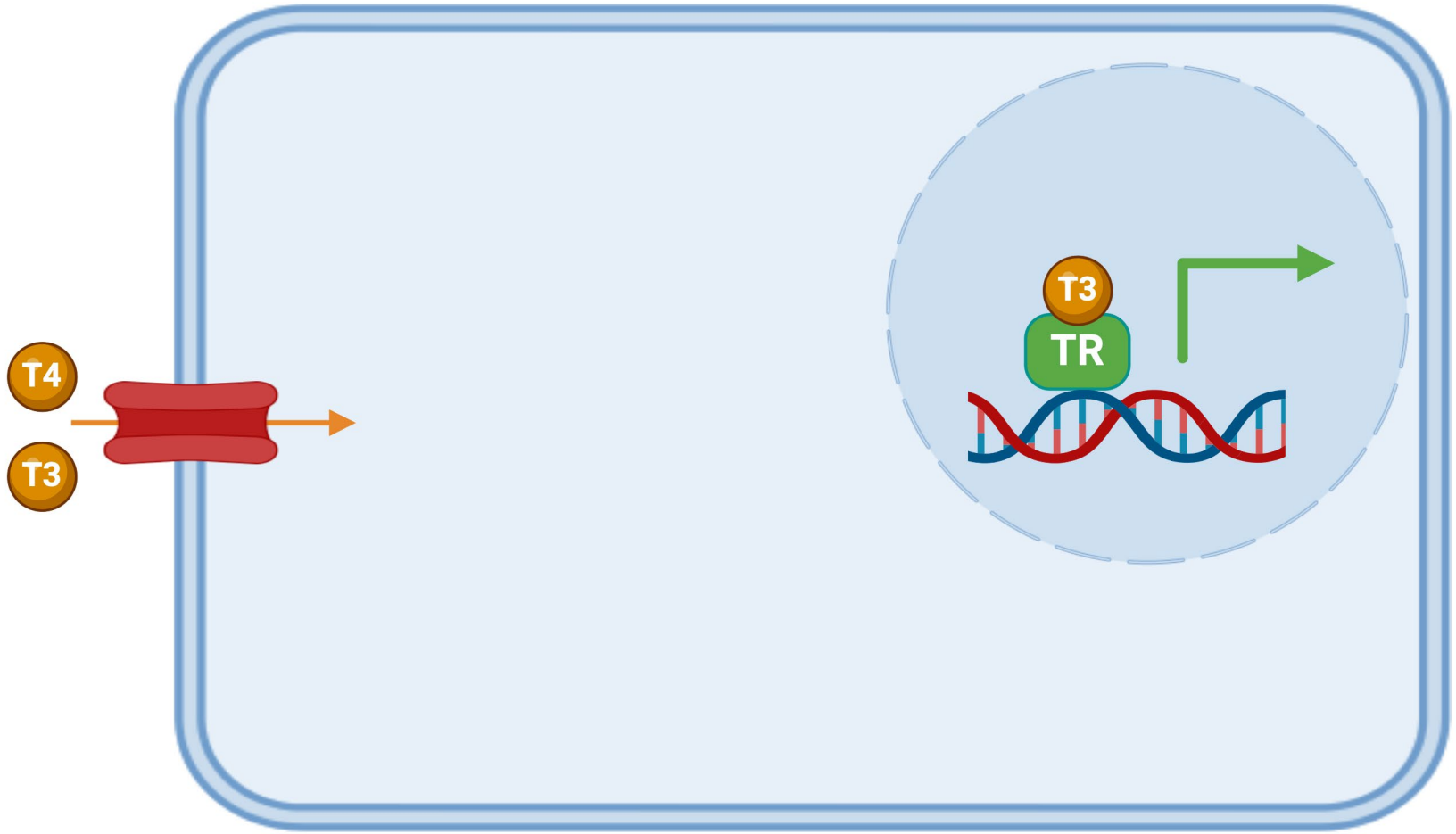
T3 target cell: hormones enter by transporter proteins



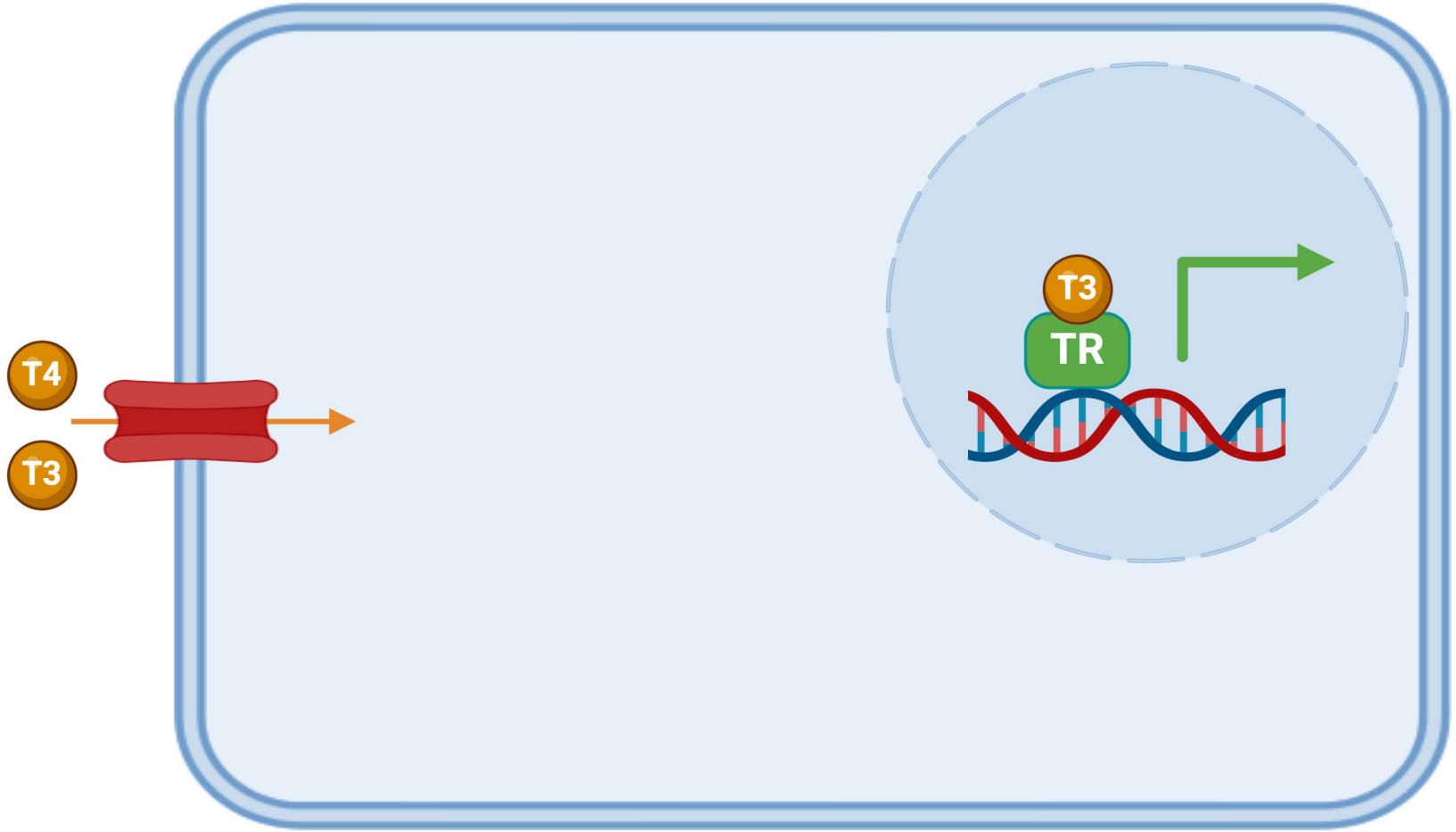
T3 regulates developmental and metabolic process via its receptor



MCT8



Thyroid hormone signaling disorders



MCT8

MCT8 deficiency

TRα/TRβ

RTHβ

Outline

Brief context of thyroid hormone signaling

MCT8 deficiency: key features & mechanisms of disease

Triac Trial I

Real world data

QoL, survival data & Triac Trial II

MCT8 deficiency: developmental & metabolic disorder

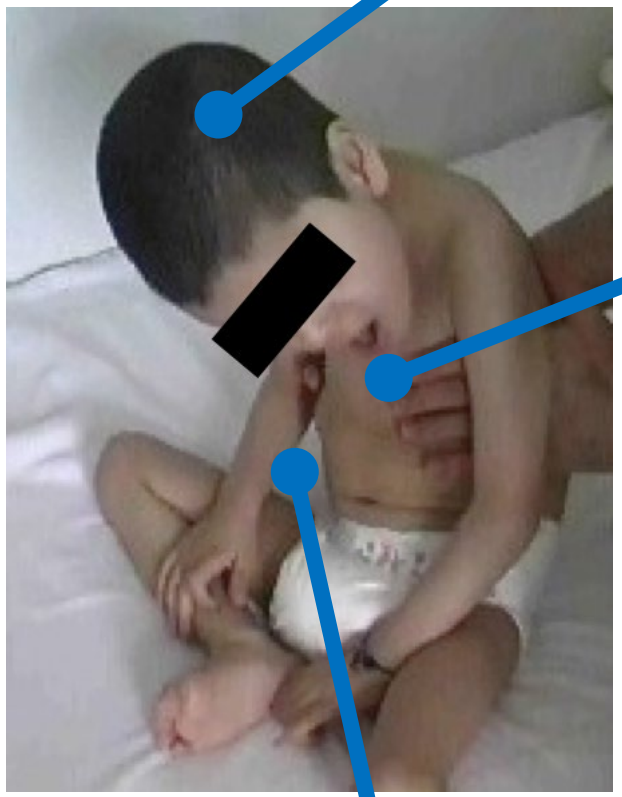
MCT8 deficiency: developmental & metabolic disorder

MCT8 deficiency
International registry for rare thyroid disorders

T4 ↓
T3 ↑
TSH =

Epilepsy

Sleep disturbance



No head control

Hypotonia

Hypokinesia

Low body weight & feeding problems

Tachycardia

Frequent infections

Dystonia

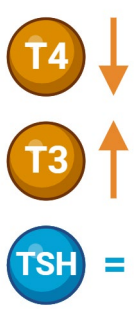
Scoliosis

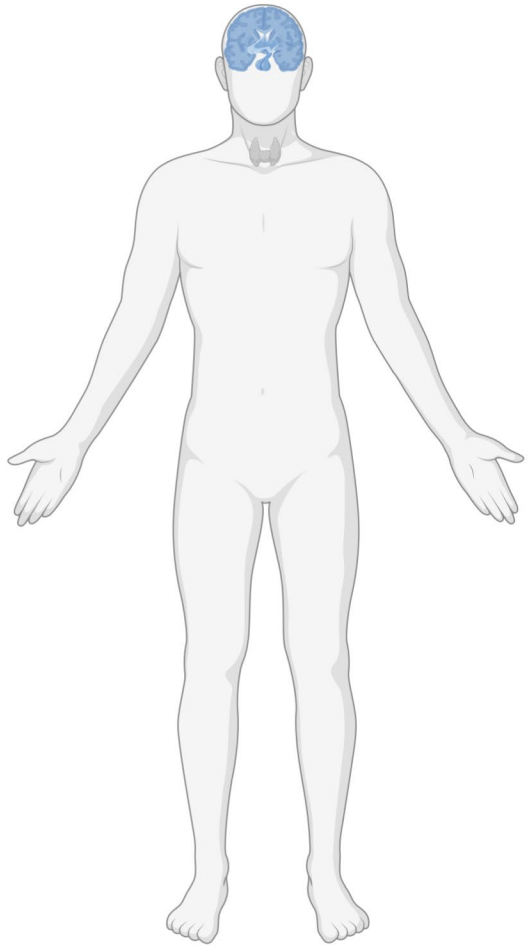
Low muscle mass

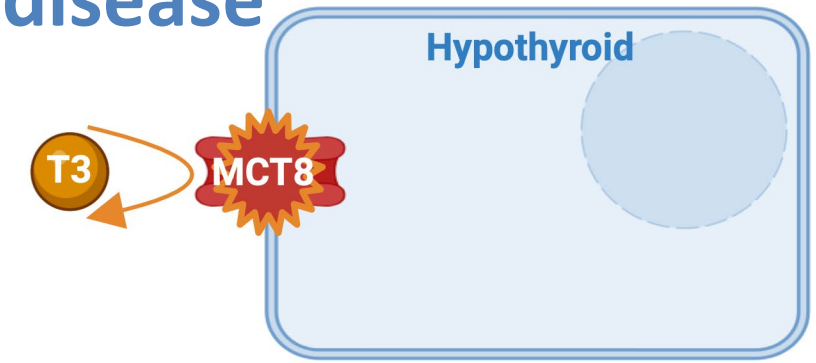
Wheel chair bound

Friesema, Lancet 2004; Dumitrescu, AJHG 2004; Groeneweg, Lancet D&E 2020

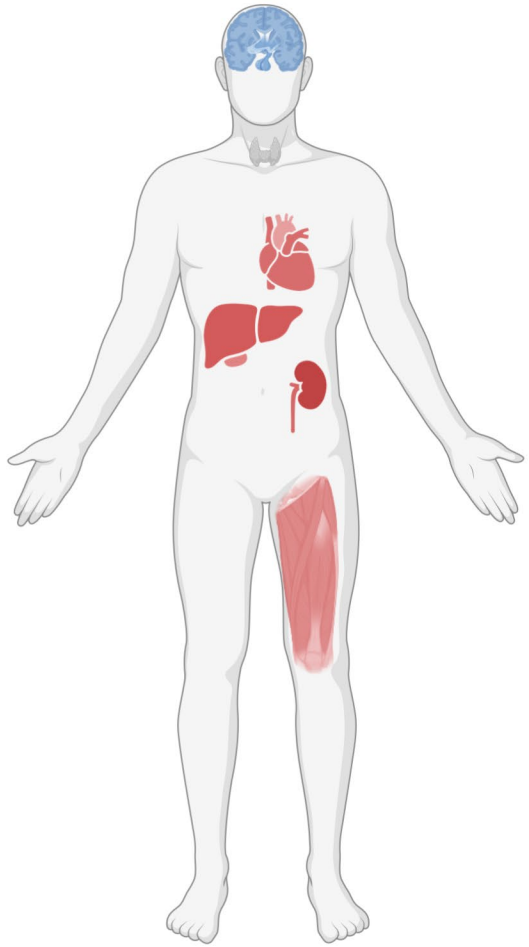
MCT8 deficiency: mechanisms of disease

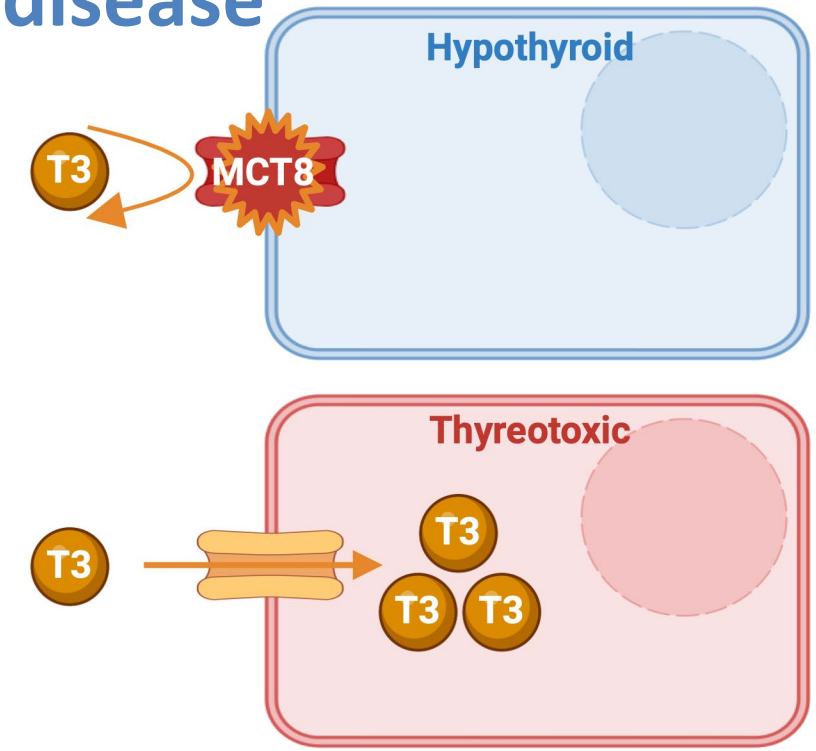




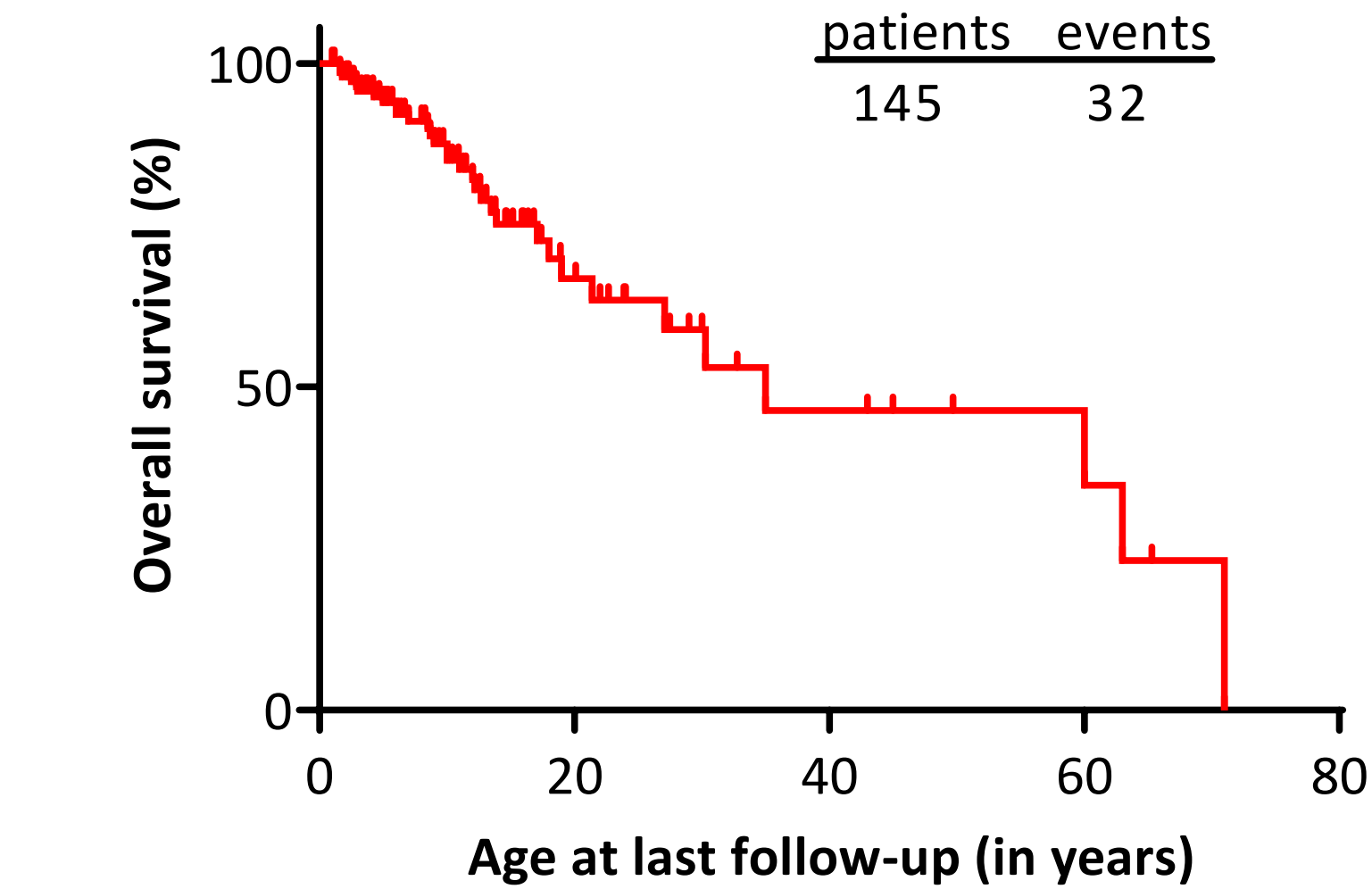


MCT8 deficiency: mechanisms of disease



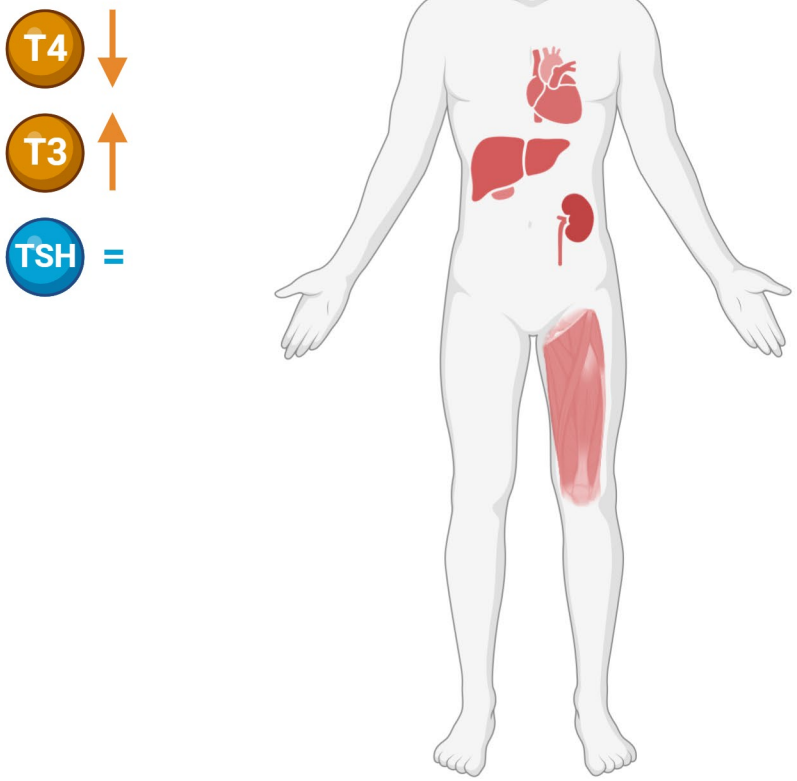


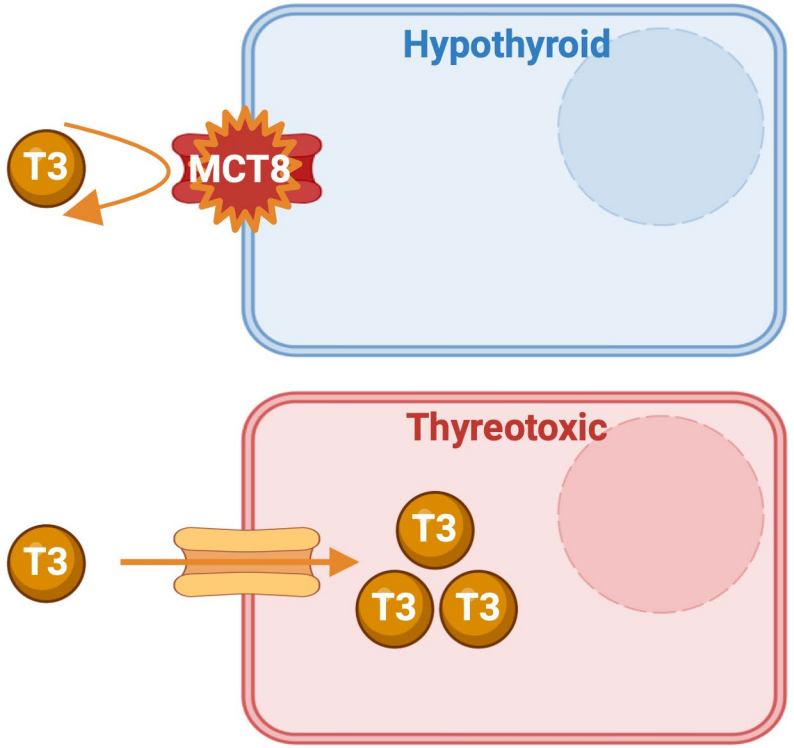
High mortality rate



Groeneweg, Lancet Diab Endocrinol 2020

Therapy: dual action

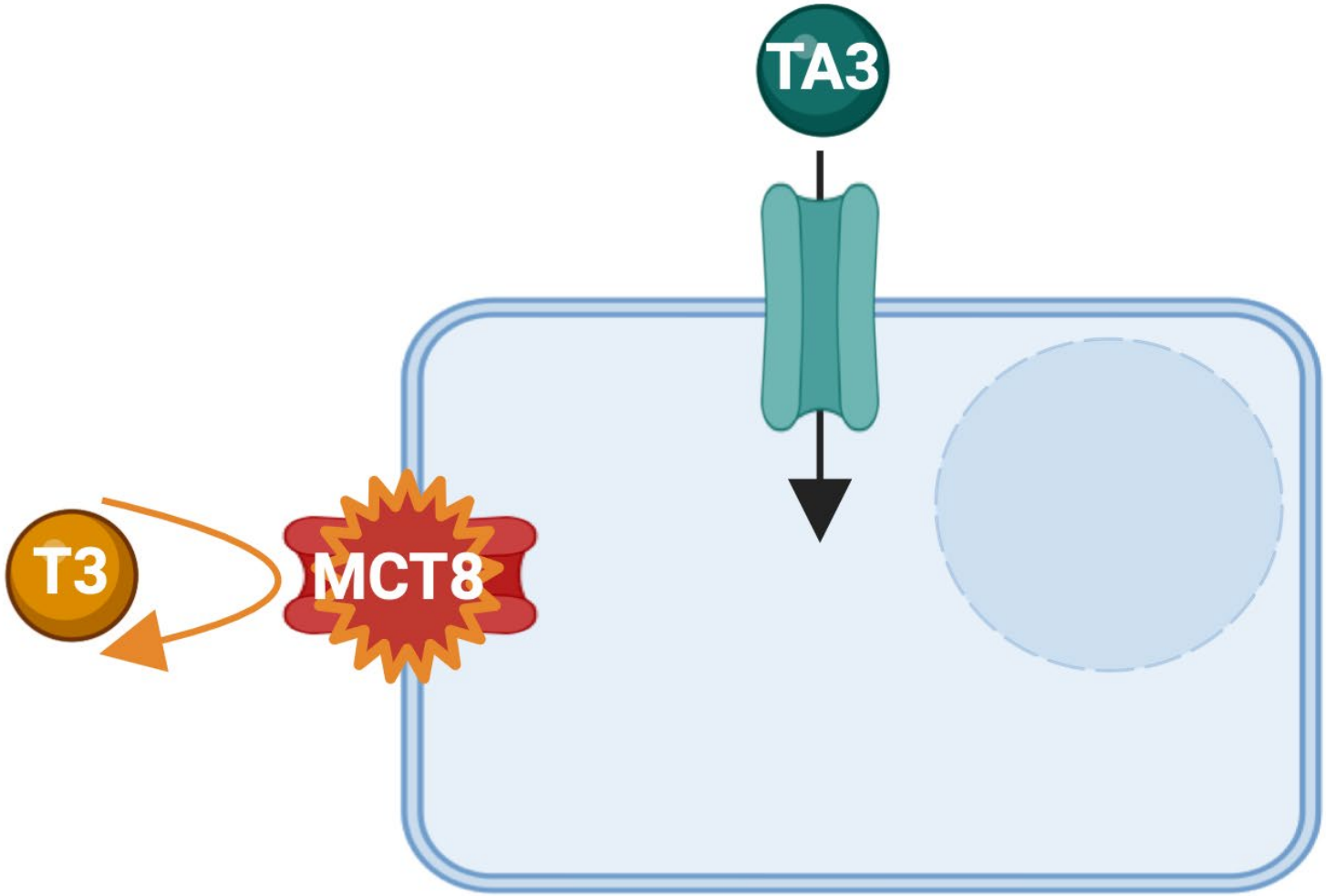


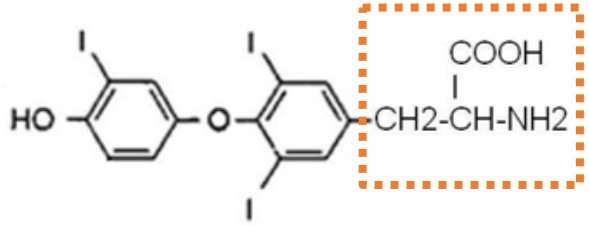


Increase thyroid hormone action

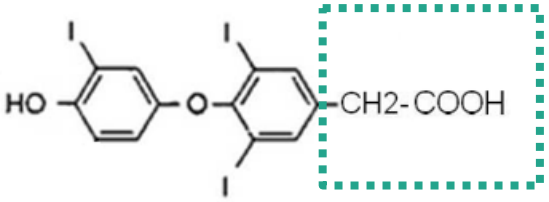
Reduce thyroid hormone action

T3 analog tiratricol (Triac) – principle in MCT8 defective cells



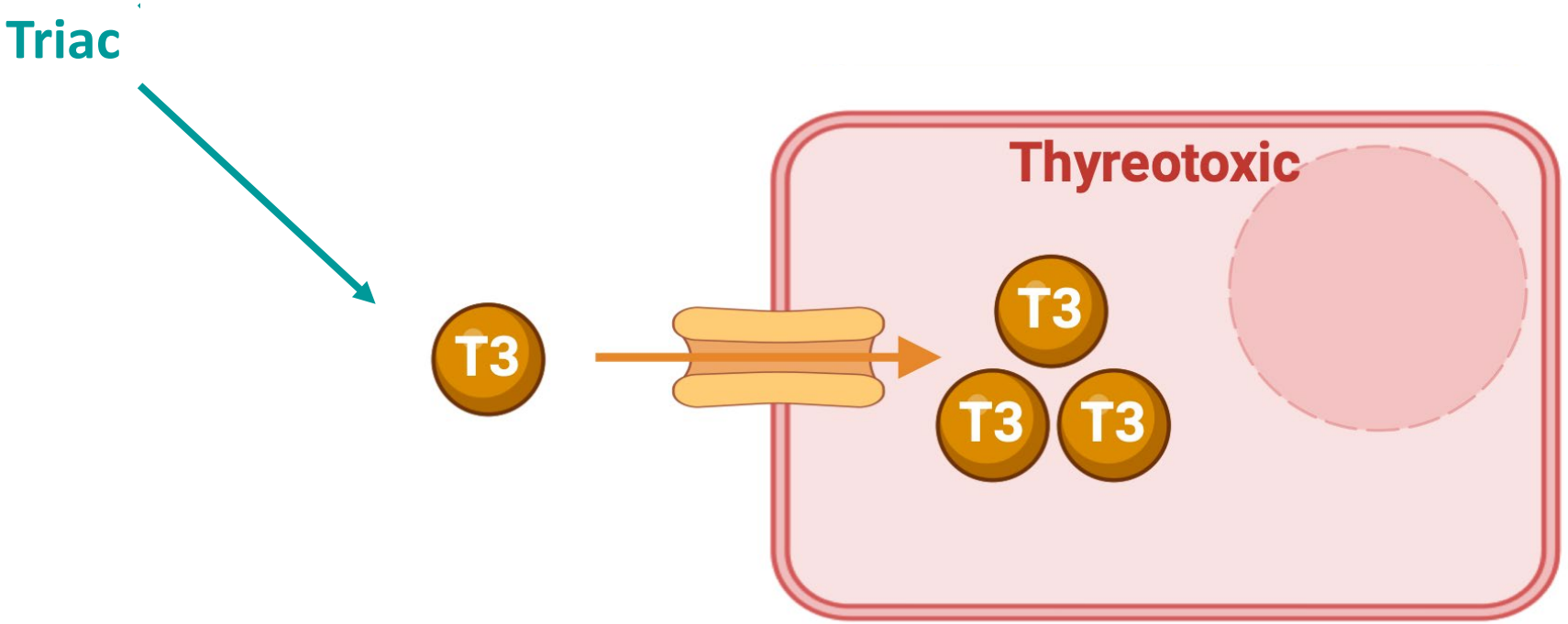


T3



Triac (TA3)

Is Triac effective in patients with MCT8 deficiency?



Metabolic phenotype

Triac Trial I

Outline

Brief context of thyroid hormone signaling

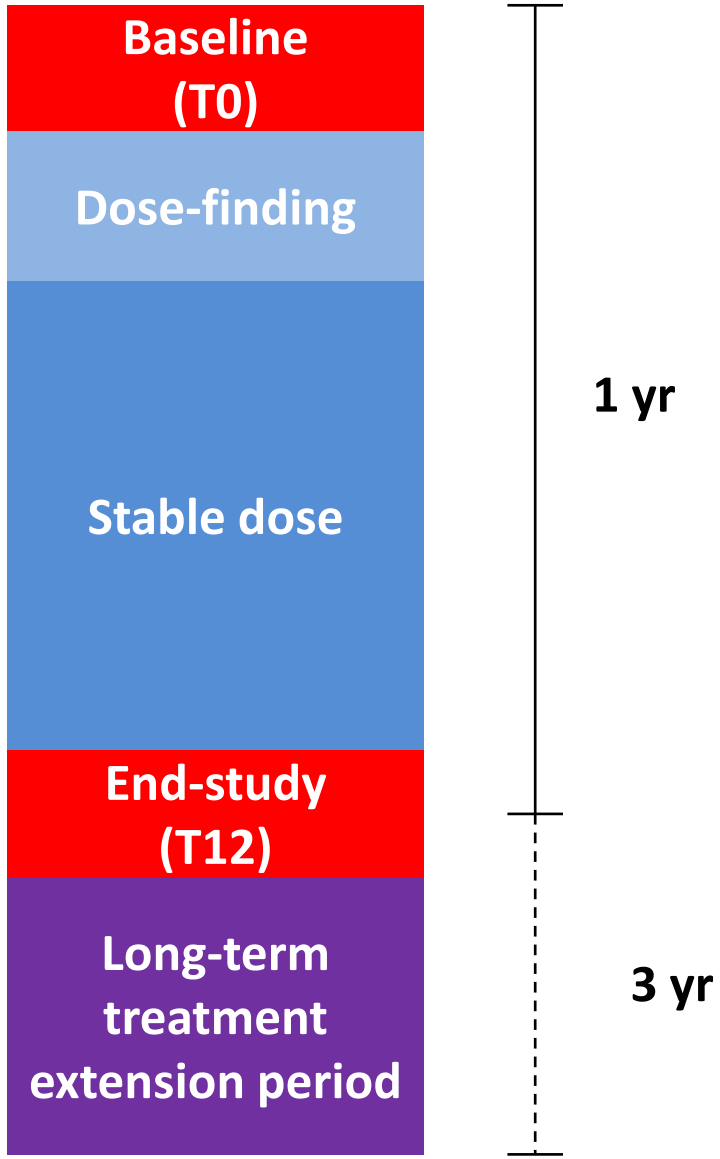
MCT8 deficiency: key features & mechanisms of disease

Triac Trial I

Real world data

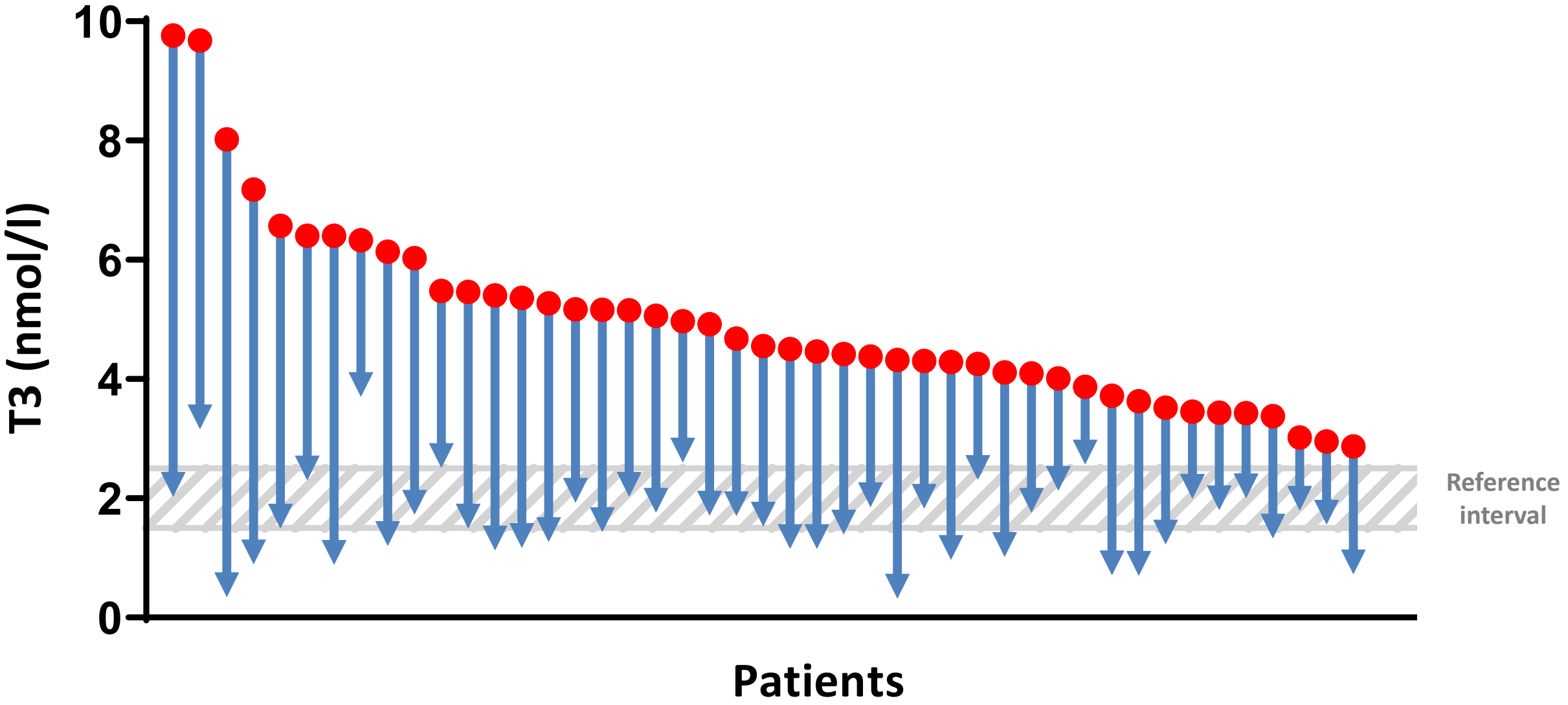
QoL, survival data & Triac Trial II

Triac Trial I: international phase 2 trial



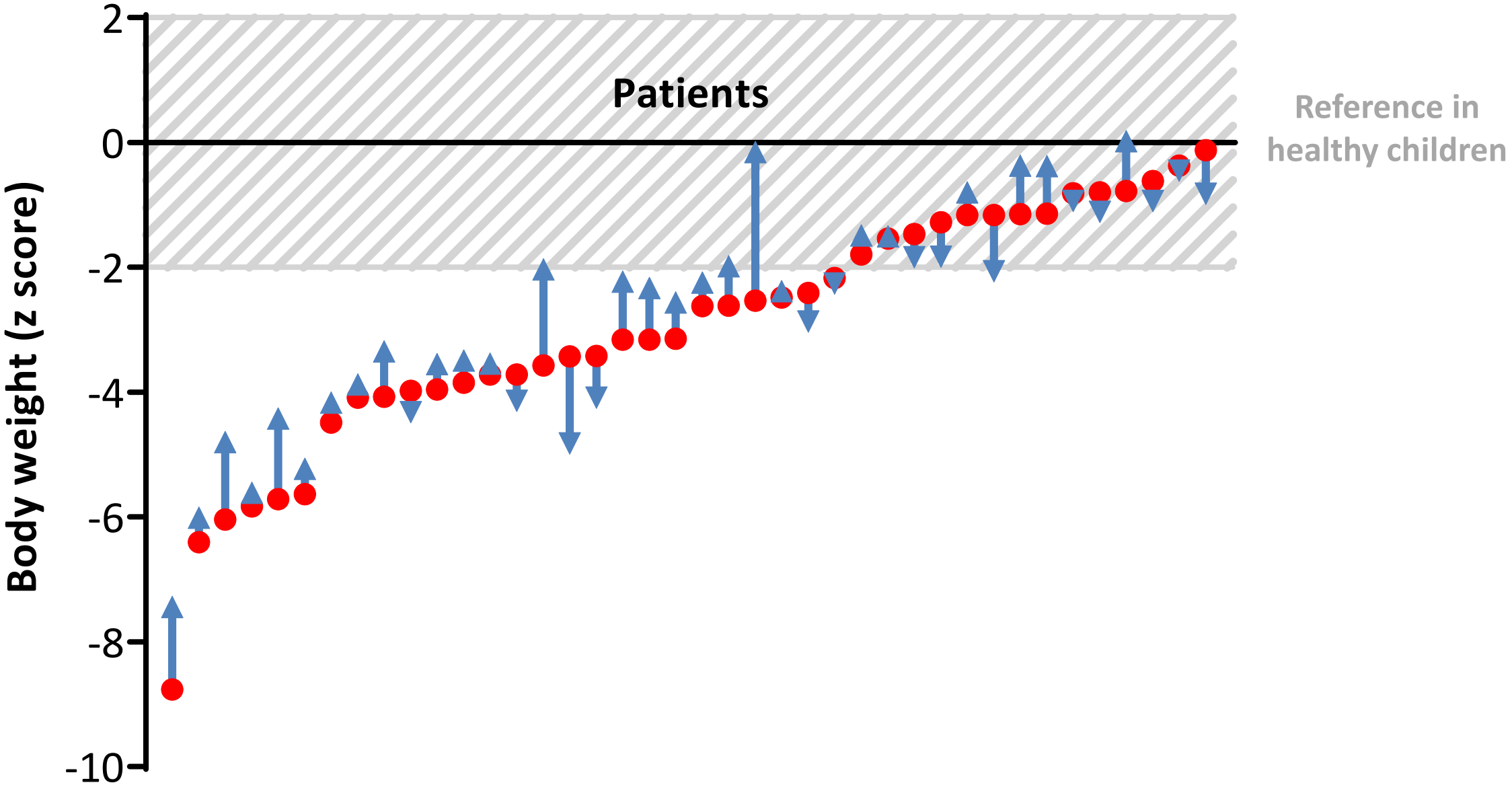
N=46
Median age 7.1 yrs
(range 0.8 – 66.8)

Primary outcome: T3 concentrations normalize



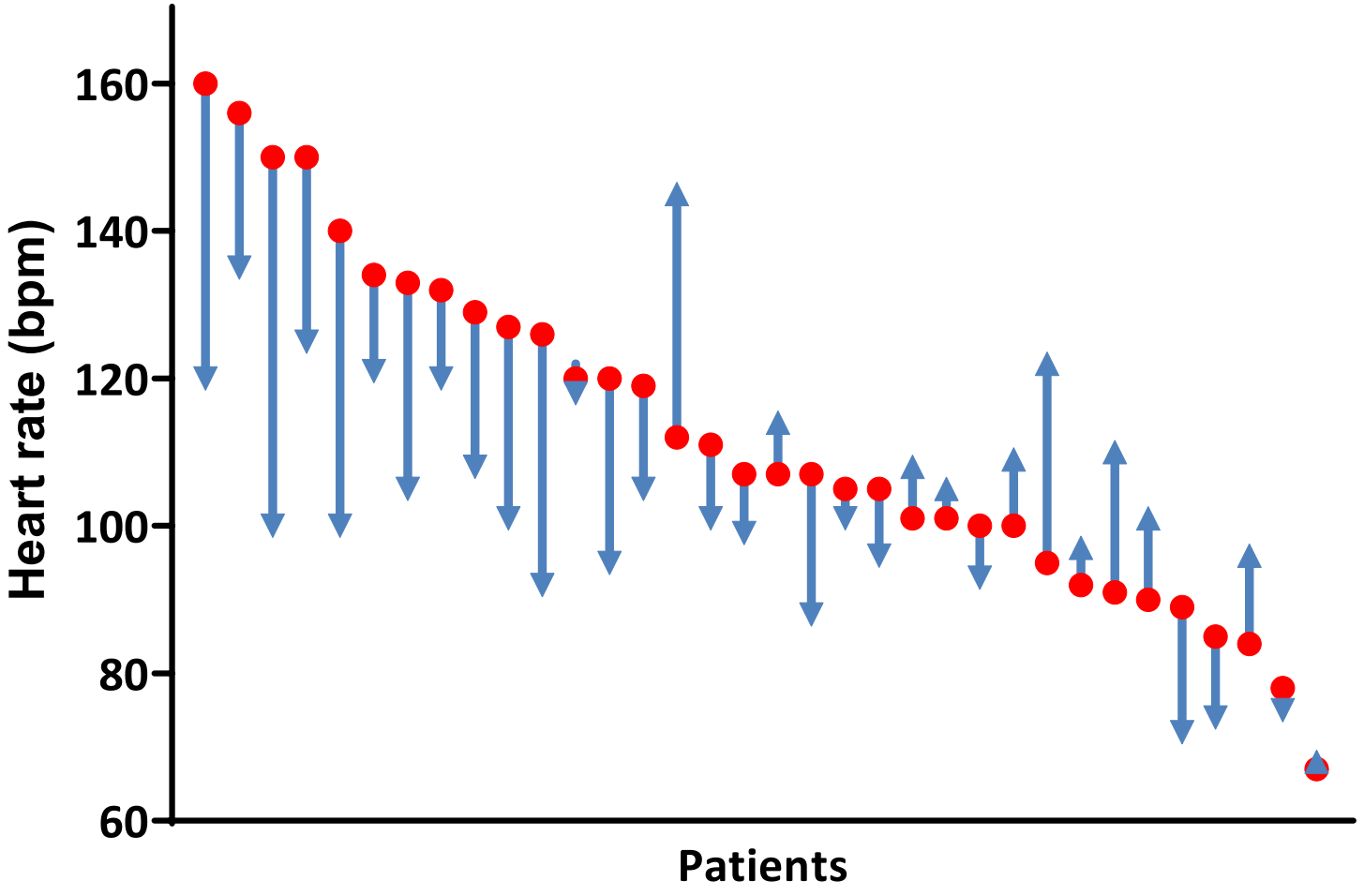
Groeneweg, Lancet Diab Endocrinol 2019

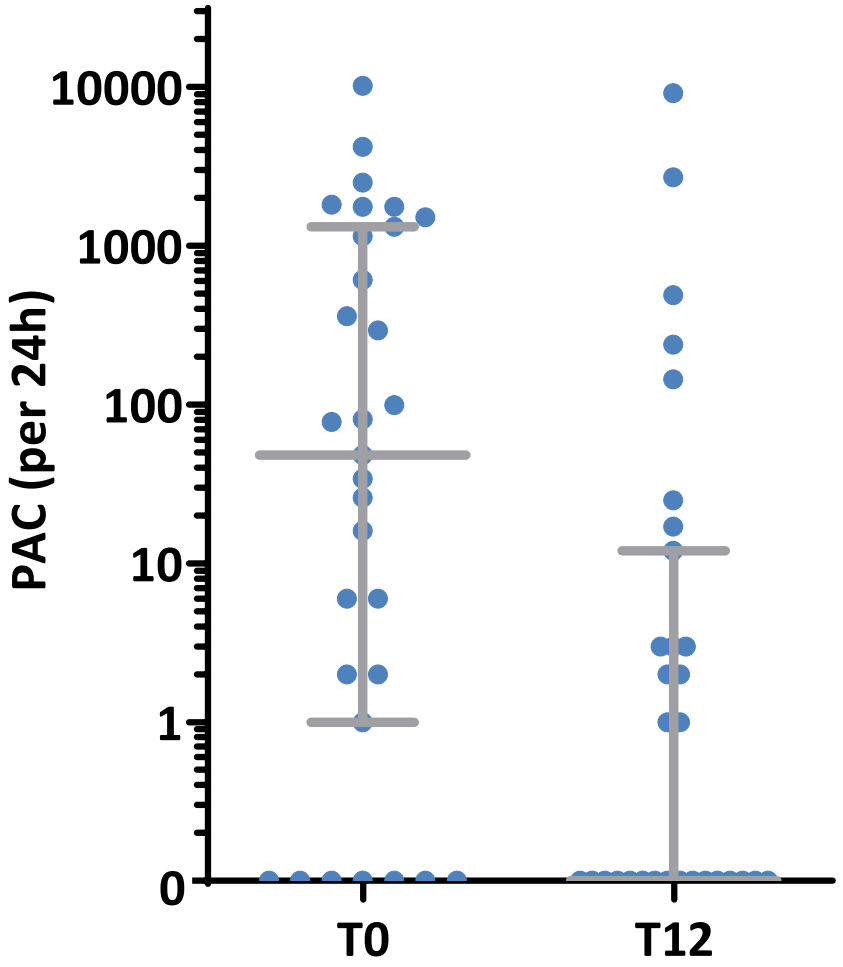
Secondary outcomes: body weight improves



Groeneweg, Lancet Diab Endocrinol 2019

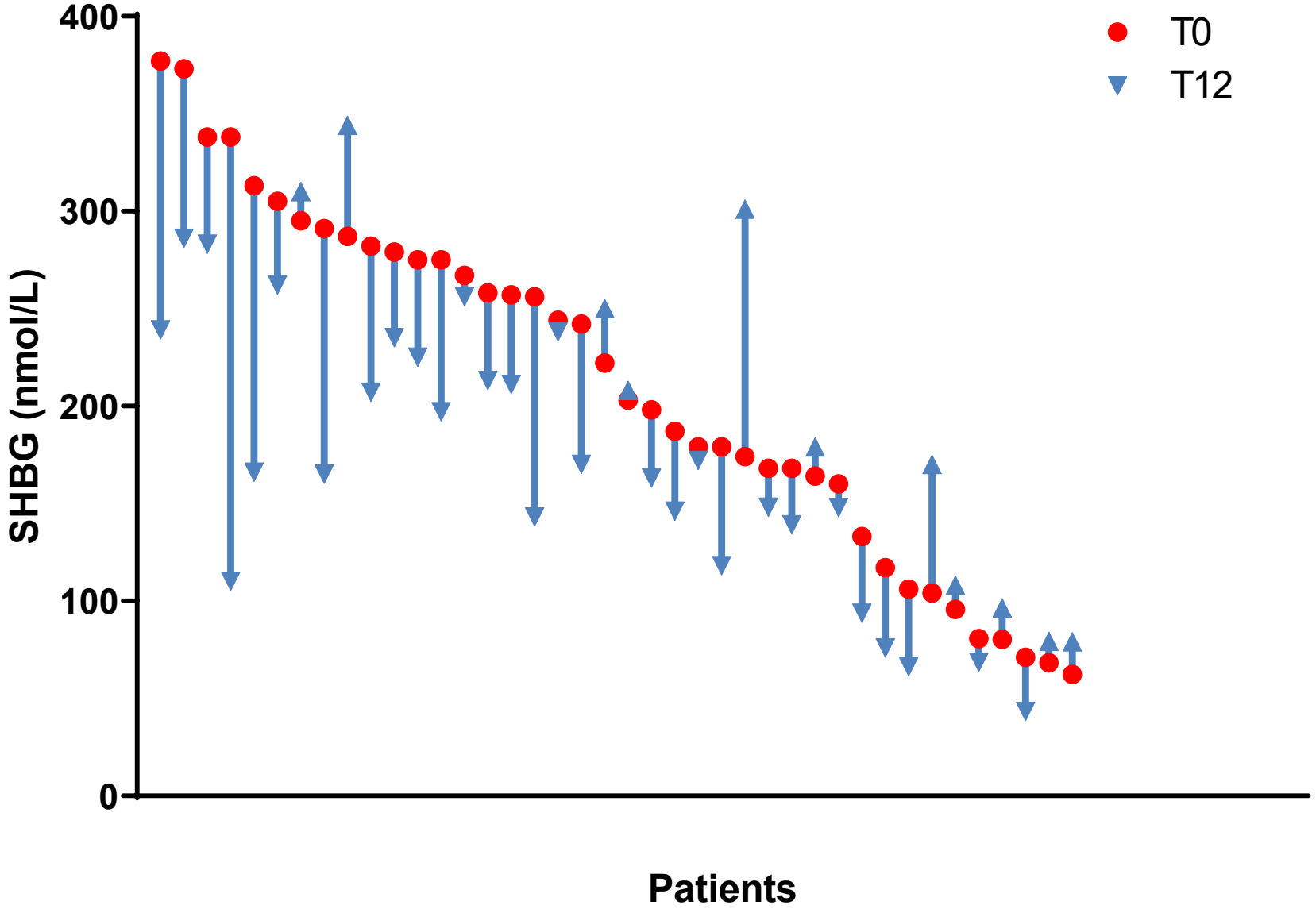
Secondary outcomes: heart rate improve & PACs subside





Groeneweg, Lancet Diab Endocrinol 2019

Secondary outcomes: biochemical markers improve



Groeneweg, Lancet Diab Endocrinol 2019

Outline

Brief context of thyroid hormone signaling

MCT8 deficiency: key features & mechanisms of disease

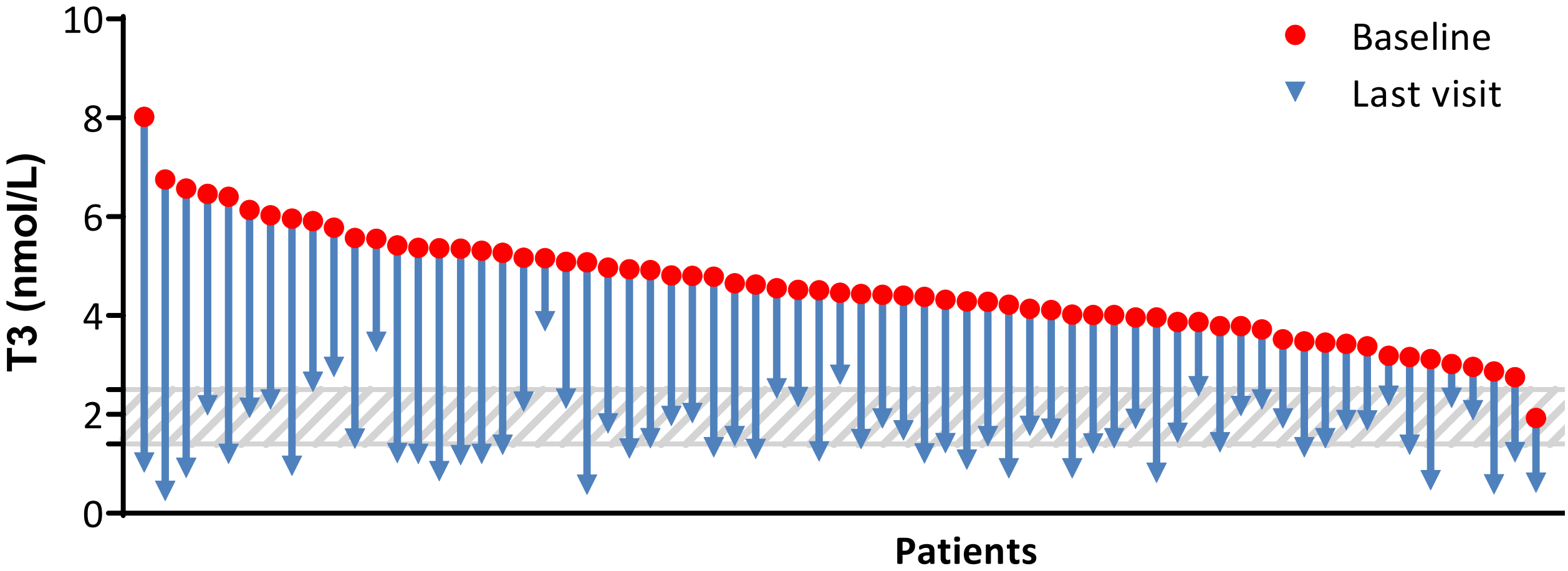
Triac Trial I

Real world data

QoL, survival data & Triac Trial II

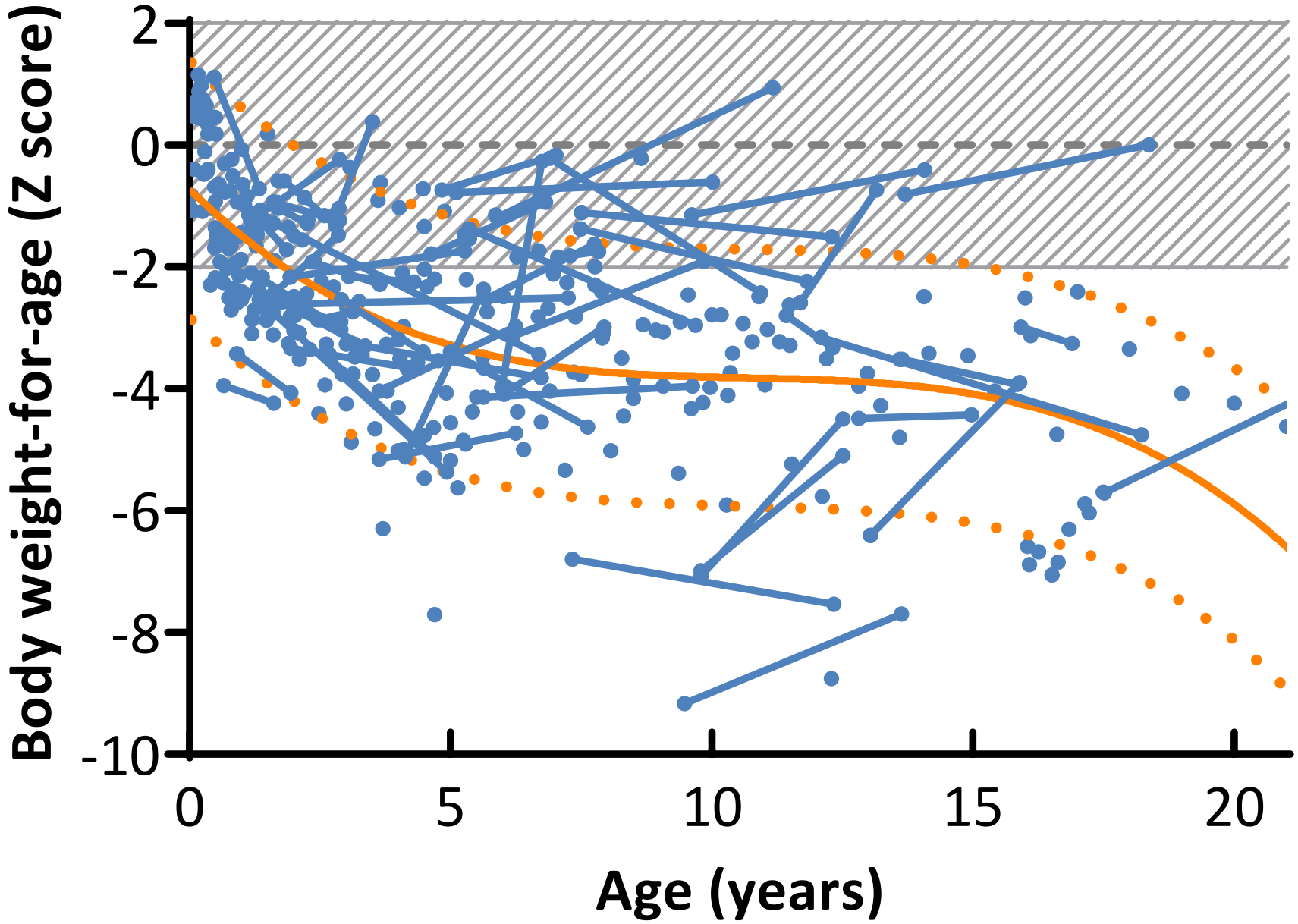
Triac: real world data

Real world data: long-term reduction T3



Van Geest, JCEM 2022

Real world data: sustained improvement of body weight



Reference in healthy children

Natural history in untreated MCT8 deficiency

Van Geest, JCEM 2022

Outline

Brief context of thyroid hormone signaling

MCT8 deficiency: key features & mechanisms of disease

Triac Trial I

Real world data

QoL, survival data & Triac Trial II
(boring slides)

Triac: effects on QoL

Post-hoc analyses on caregiver-reported patient-centered outcome measures from TT1

Semi-structured interviews (baseline, F-U visits, EoS visit in n=40) on complex needs and daily care challenges

Most prominent changes
Positive: improved interaction (22/39), improved alertness (19/39), improved motor skills (12/39), improved sleep (8/39)
Negative: Increased constipation, increased unsettledness (1/39)

Less perspiration (8,1% vs 48,6, EoS vs baseline)

40/40 preferred to continue Triac treatment

Triac: effects on mortality

International multi-center cohort study (n=173 sites; n=48 countries; n=484 screened patients)

Excluded patients (DOB < 2004, n=152; limited data, n=66; unknown LoF, n=36)

Baseline characteristics with (n=111) or without (n=117) Triac similar
(except untreated patients less in Western countries)

Median F-U: 4.8 yrs (IQR=2.7-8.4); 5 deaths in treated, 27 in untreated group

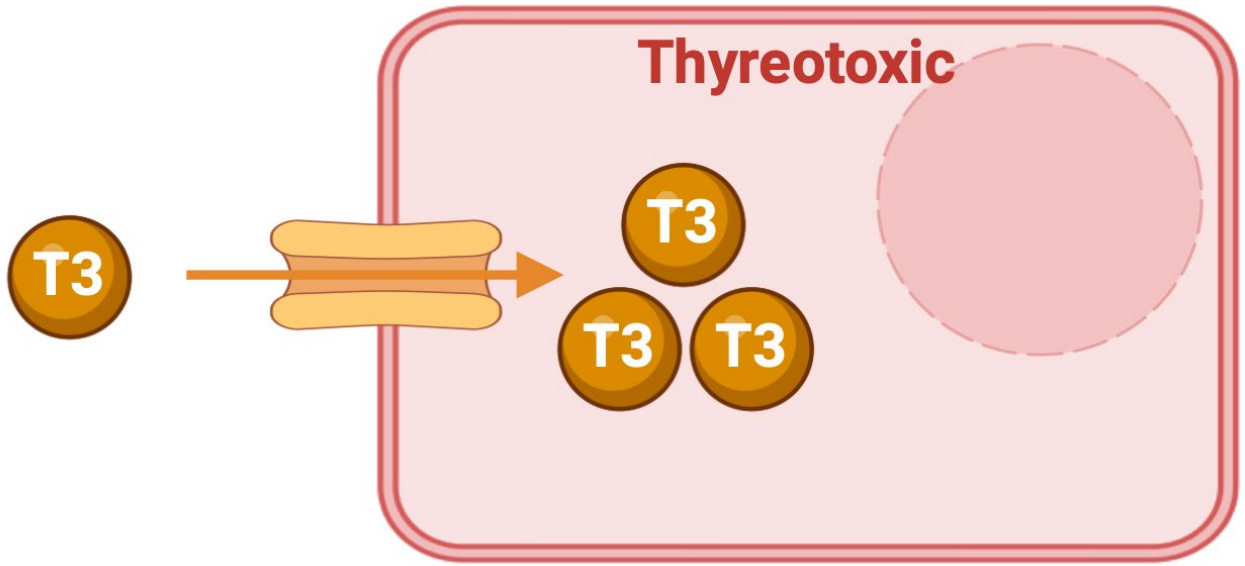
Triac treated patients had ~ 3-times lower risk of all-cause mortality
(HR=0.28, 95%CI=0.09-0.91, $p<0.05$)

Ongoing analyses: confirm robustness; increase number of patients

Is Triac effective in patients with MCT8 deficiency?

Triac

Yes



Metabolic phenotype

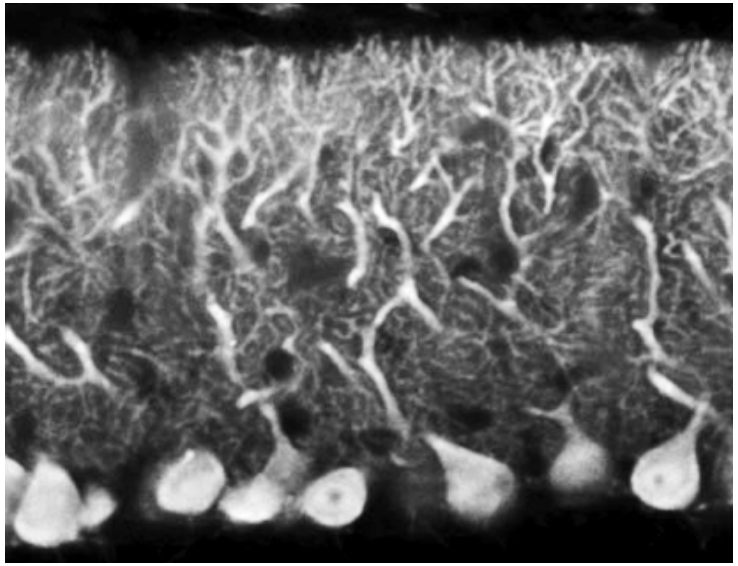
Triac Trial I

Real world data

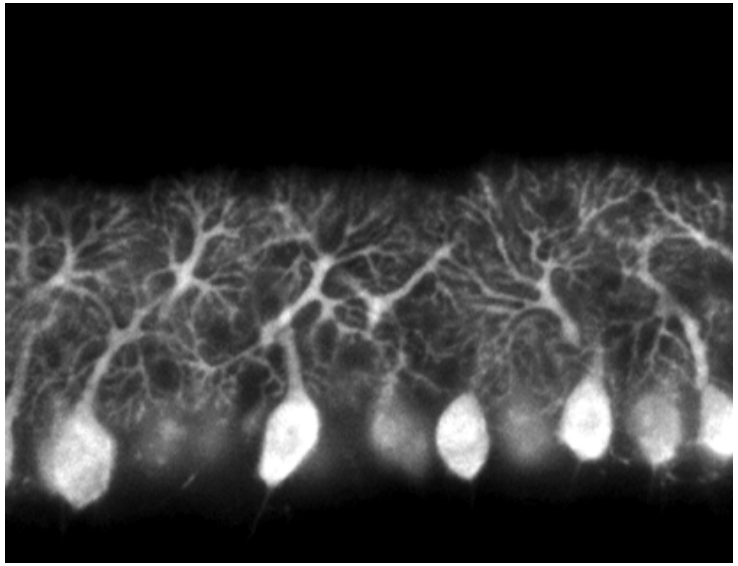
Triac Trial II - background

Triac normalizes brain development in mouse model

WT

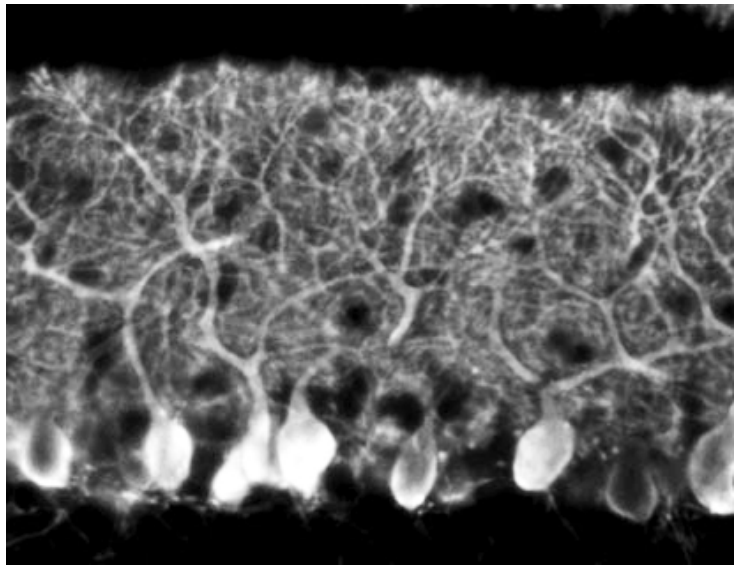


Mct8/Oatp1c1 DKO



- Triac

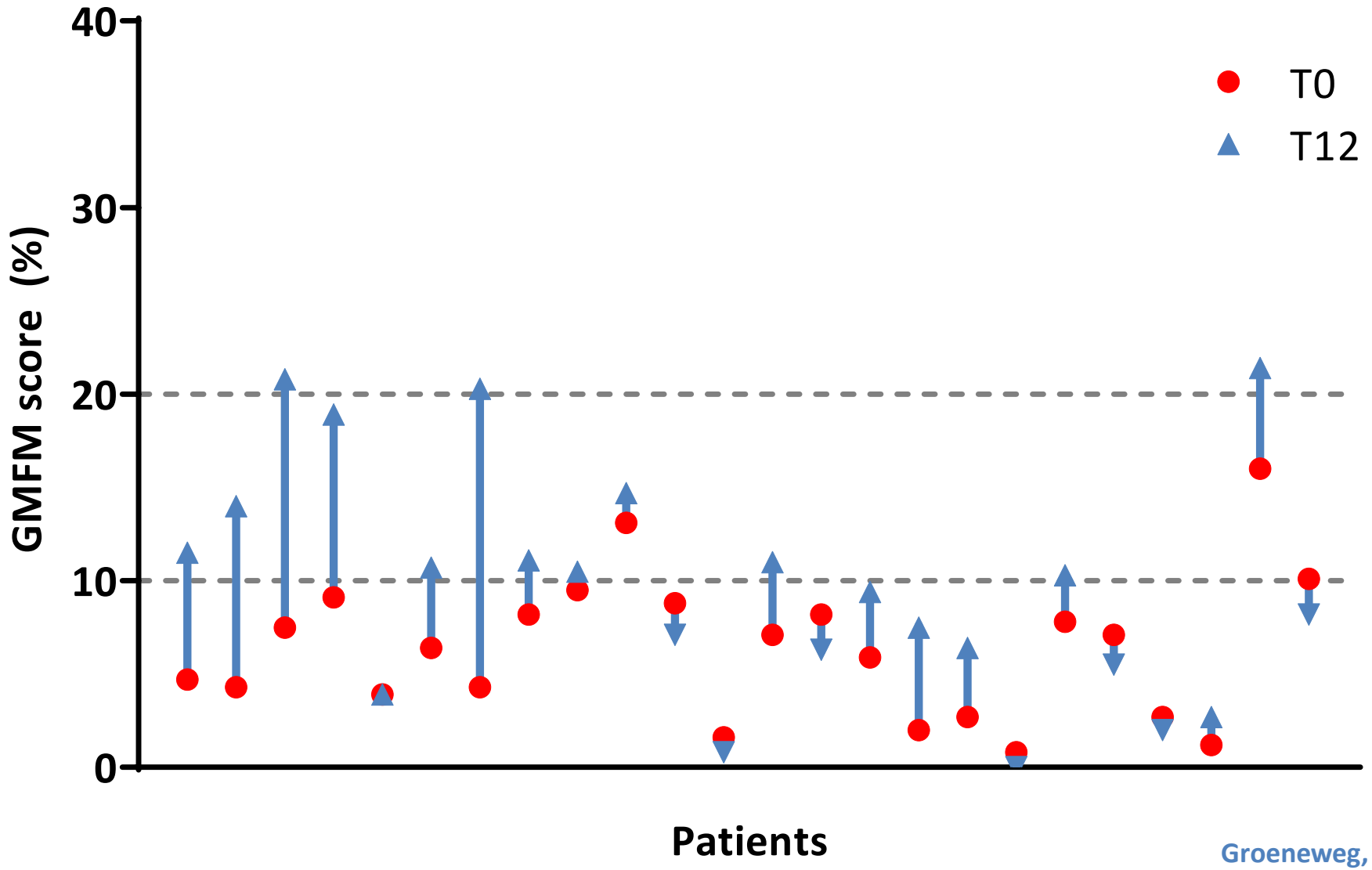
Mct8/Oatp1c1 DKO



+ Triac

Kersseboom, Mol Endo 2014

Triac Trial I – GMFM (exploratory analysis)



Groeneweg, Lancet Diab Endocrinol 2019

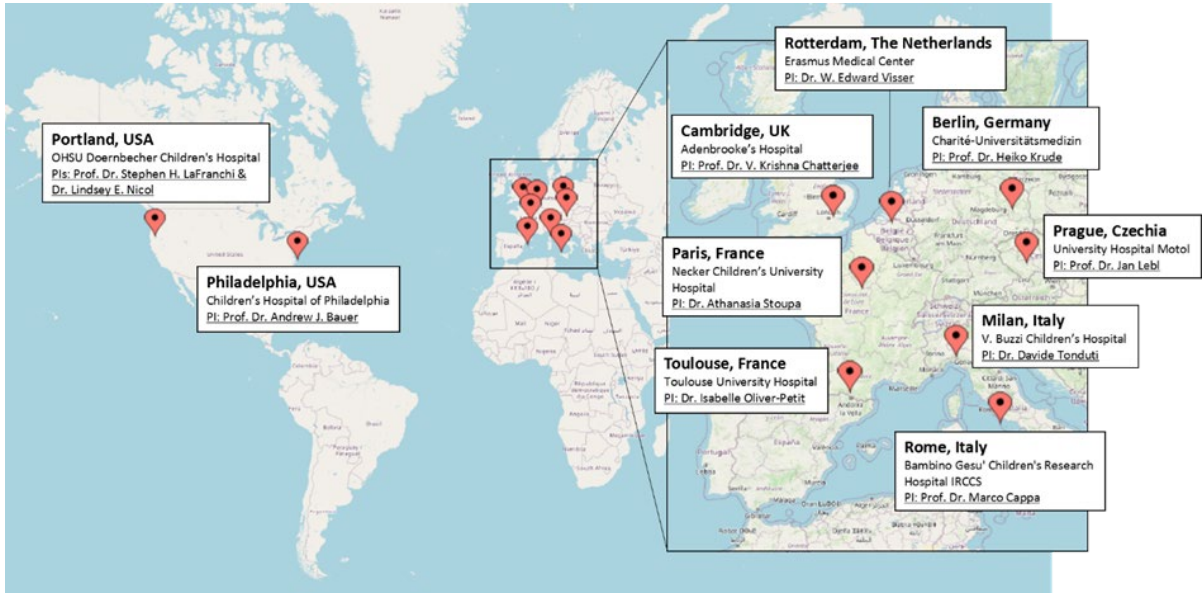
Triac Trial II – in/exclusion criteria

International multi-center open label trial

Inclusion criteria

Male patient with MCT8 deficiency

Aged ≤ 30 months at baseline



Exclusion criteria

Previous Triac treatment

Previous L-T4 and/or PTU treatment for > 3 months

Triac Trial II – outcomes

Primary outcomes
GMFM-88 total score
BSID-III Gross Motor Domain

} **Comparison with historical controls**

Secondary outcomes
Item 10 (head control) and 24 (sitting) of GMFM-88
Complete BSID-III
Motor milestone responder analysis of standardized neurological examination (HINE)
Parameters of thyrotoxicosis

Triac dosing up to 200 ug/kg/day

Triac Trial II – cohort

Screened (n=23)

Enrolled (n=22)

Finalized 96w (n=21)

Triac Trial II – primary outcomes

No statistical (and clinical) relevant change in GMFM or BSID

Triac Trial II – secondary outcomes

Strong reduction in T3 concentrations

Triac well tolerated

Triac Trial II – conclusions

No effect on brain development with Triac in early life

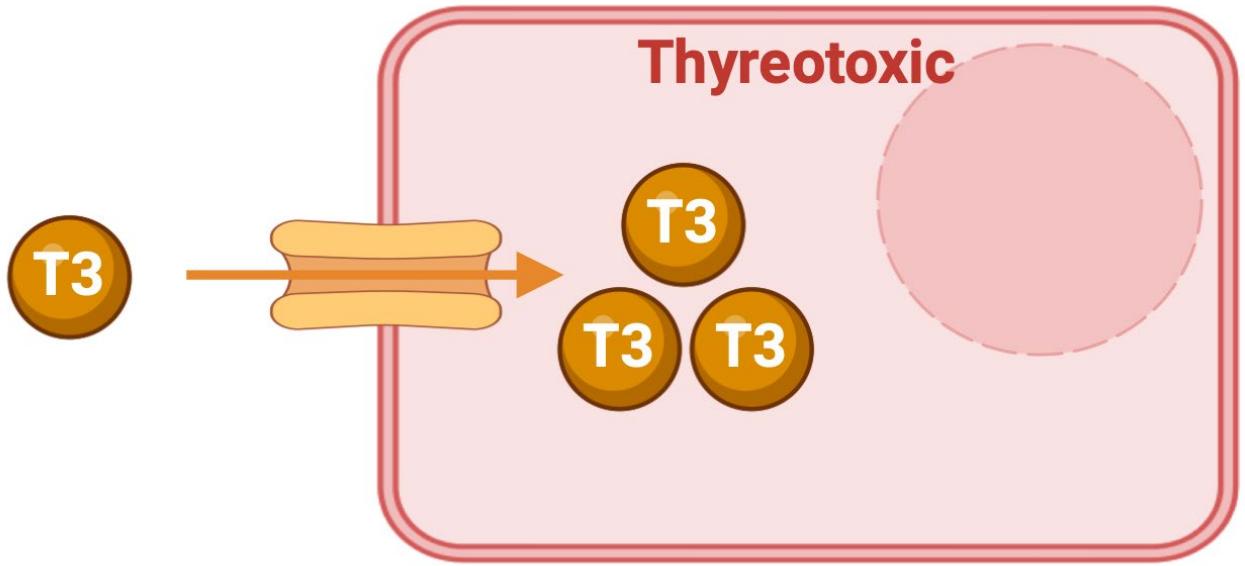
Confirmation of reduction in T3 concentrations

Ongoing analyses (subgroups)

Is Triac effective in patients with MCT8 deficiency?

Triac

Yes



Metabolic phenotype

Triac Trial I

Real world data

Take home message

MCT8 deficiency: hypothyroid & thyreotoxic features

Triac

alleviates metabolic/thyreotoxic phenotype

does not improve neurodevelopment in patients < 30 months

(full analysis in progress)

2024 European Thyroid Association Guidelines on diagnosis and management of genetic disorders of thyroid hormone transport, metabolism and action

Luca Persani[1,2,*], Patrice Rodien[3,*], Carla Moran[4,5,6,7,*], W Edward Visser[8,*], Stefan Groeneweg[8,*], Robin Peeters[8], Samuel Refetoff[9], Mark Gurnell[4], Paolo Beck-Peccoz[2] and Krishna Chatterjee[4]

Persani, ETJ 2024

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Wenjun Liao

Anna Lopez

Lizette Blankers

Merel Stegenga

Leonoor Schonebaum

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Amnon Zung

Poland

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Romania

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Diana Barca

Alice Dica

Iuliu Bacos

Spain

Alberto Alcantud

The Netherlands

Martien Manshande †

Paul Vrijmoeth

Ineke Lunsing

Jurgen Jansen

Nicole Wolf

Frank Visser

Stan Nowak

Angelique Zandstra

Nitash Zwaveling

Anne-Marie van Wermeskerken

Jet van der Spek

David Koolen

South Africa

Adri van der Walt

Turkey

Serap Turan

United Kingdom

Krishna Chatterjee

Carla Moran

Greta Lyons

Anne McGowan

Yogen Singh

Paul Dimitri

Jonathan Gallichan

USA

Amy Lawson Yuen

Cheyenne Dewey

Andy Bauer

Stephen LaFranchi

Animal studies

Essen, Germany: Heike Heuer

EGETIS THERAPEUTICS

WE CARE

FOR THE RARE

Investor Day

December 18, 2024

Q&A

EGETIS THERAPEUTICS

Global Launch Preparations

December 18, 2024

Henrik Krook
VP Commercial Operations

Focused on Broad Patient Access and Value Creation

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

Reimbursement & physicians prescribing

EXAMPLES OF KEY ENABLERS

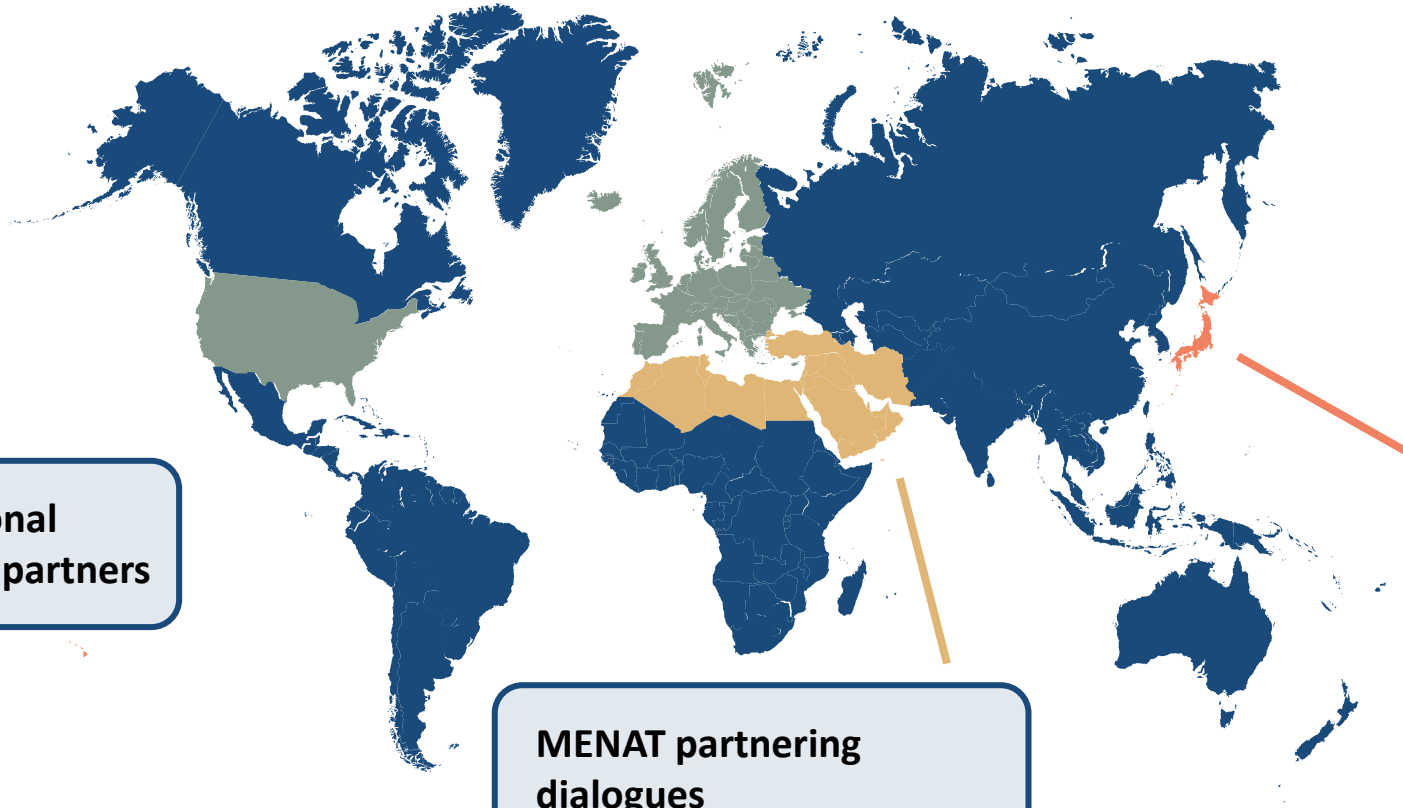
Disease awareness including educational initiatives

Deliver solid value proposition to key stakeholders

Preparing *for Emcitate* launch by Egetis and partners

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

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



Optimizing additional countries through partners

MENAT partnering dialogues

Japan license deal with Fujimoto

Launch possible with lean & agile team

Unique setting for *Emcitate* in MCT8 deficiency

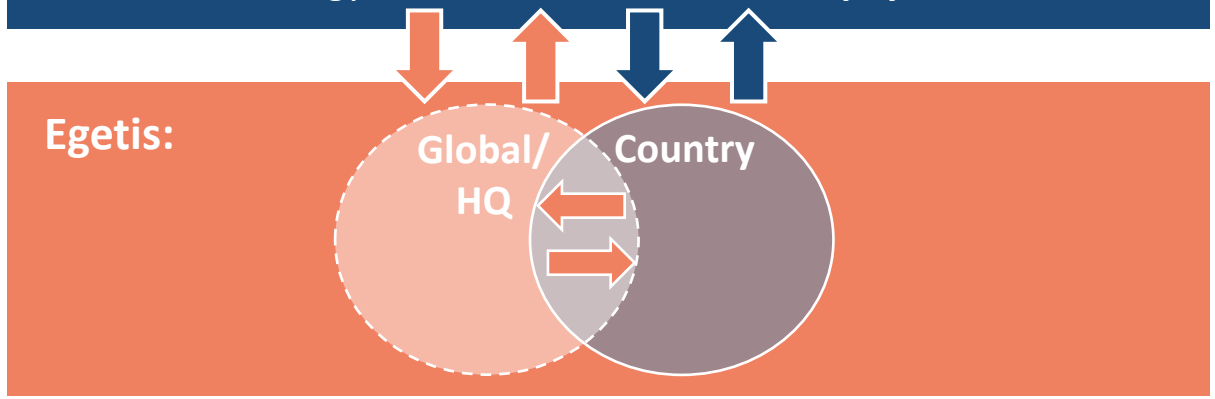
- Addressing unmet medical need
- No competition
- KOL support
- Treatment guidelines
- Global community

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**



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

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

European Thyroid Association recommends tiratricol (Emcitate®) as long-term therapy for all patients with MCT8 deficiency in new guidelines

July 17, 2024

Stockholm, Sweden, July 17, 2024. Egetis Therapeutics AB (publ) ("Egetis" or the "Company") (Nasdaq Stockholm: EGTX), today announced that the European Thyroid Association (ETA) has published new guidelines recommending the use of tiratricol (TRIAC or Emcitate®) as long-term therapy for all patients with MCT8 deficiency, and for certain patients with Resistance to Thyroid Hormone (RTH)-beta, as further outlined in the guidelines.

There are currently no approved treatments for MCT8 deficiency or RTH-beta. Egetis has obtained orphan drug designation for tiratricol for the treatment of MCT8 deficiency and RTH-beta in the EU and the USA, and has submitted a marketing authorisation application in the EU, which is currently under review by the European Medicines Agency.

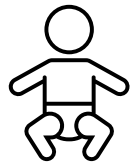
These inaugural *2024 European Thyroid Association Guidelines on diagnosis and management of genetic disorders of thyroid hormone transport, metabolism and action* were commissioned by the Executive Committee of the ETA and developed by an independent team of experts. The guidelines can be accessed here:

https://etj.bioscientifica.com/view/journals/etj/aop/etj-24-0125/etj-24-0125.xml

Emcitate supplied globally in managed access programs

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

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

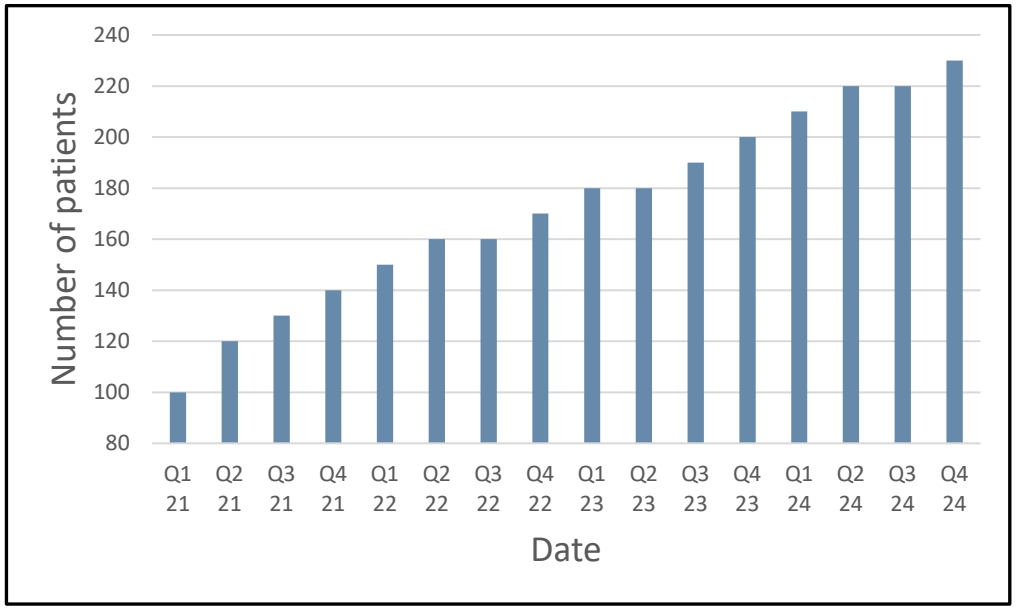


Patient

Prescriber

National Approval

Patients Receiving Emcitate in Managed Access Programs



Step-wise building team to execute on key activities at the right time for launch success

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

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

Henrik Krook, SE
VP, Commercial Operations

ALEXION

Henna Oittinen Corbinelli, CH
Medical Director Europe & International

BAYER

Nadia Georges, CH
Global Head, Market Access & Pricing

SANOFI

Susana Roche, FR
Associate Director Global
Medical Affairs Operations

argenx

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

ACTELION

Anny Bedard, US
President Egetis North America

Shire

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

Otsuka

Azza Trad, FR
GM France

sobi
Pioneer in Rare Diseases

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

BIOMARIN

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

VERTEX

Driving disease awareness, including educational initiatives, to support diagnosis of affected patients

Meetings with MCT8 community

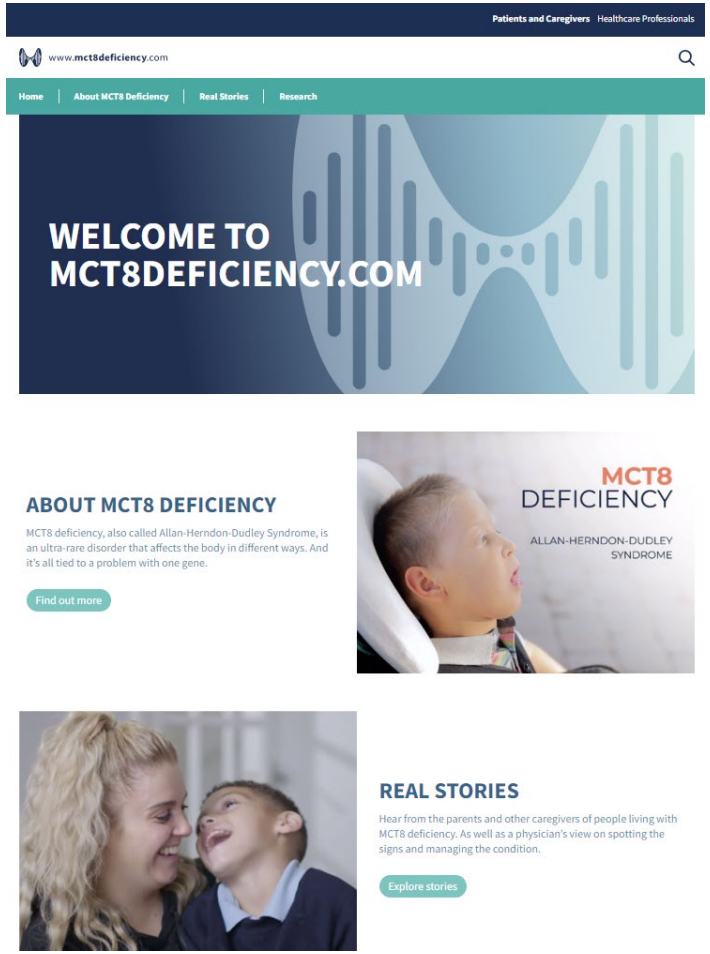
- Advisory boards
  - Caregivers
  - Medical Experts
- Congresses
- Regular meetings with physicians

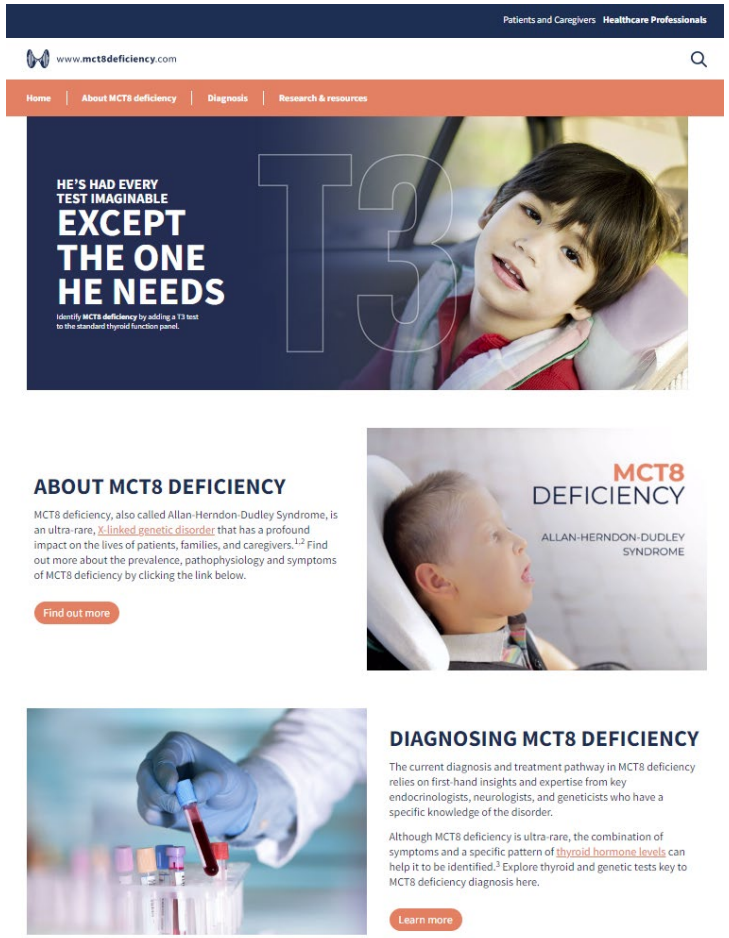
Digital channels for broader reach

- Website
- Social media
- Email campaigns
- Electronic Continuous Medical Education

mct8deficiency.com is our central disease awareness hub

Raise awareness around importance of fast and accurate diagnosis of MCT8 deficiency to ensure optimal care and management





Key assets hosted on the platform:

- 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

Expanding disease awareness momentum

Amplified by External Efforts

Constructive dialogues at scientific congresses

Scientific community generating more data

Example from Annual Meeting of the European Thyroid Association

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

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

5 additional abstracts related to MCT8 deficiency

Great work ongoing by several patient advocacy groups

MCT8 - AHDS Foundation

MCT8 Forschung e.V.

SHERMAN FOUNDATION

EURORDIS RARE DISEASES EUROPE

Thyroid Federation International

European Reference Networks #ERNcare4Ua Rare Diseases Doctors

NORD National Organization for Rare Disorders

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

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

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

High burden of MCT8 deficiency

Recently further supported by Egetis sponsored Caregiver study*


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


Quality of Life Impact of Caregiving for Patients with MCT8 Deficiency: Results From a Cross-Sectional Survey

Significant unmet medical need

Currently no drug developed and regulatory approved for MCT8 deficiency

Emcitate benefit validated by physicians and regulators

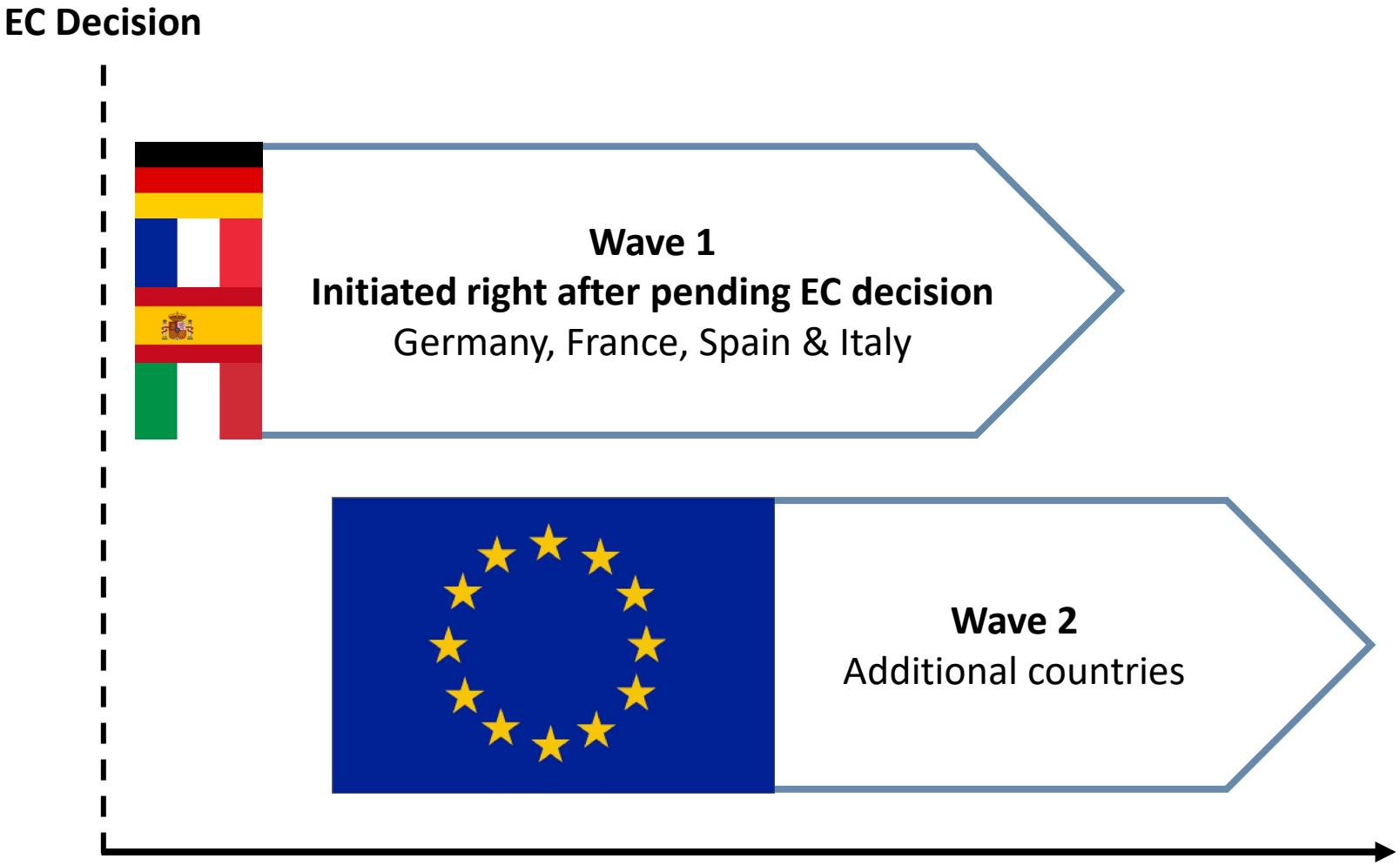
The existing clinical experience and data contributed to:

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

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

Phased EU launch: Germany first

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



Timelines P&R process for similar ultra-orphans

Germany: 1 year, but *immediate funded access in parallel*

France, Spain, Italy & rest of EU: 1-2 years

Summary

Broad patient access and value creation – building sustainable rare disease company

- Preparing for launch in Europe and US by lean and agile Egetis team, other regions through partners
- Disease awareness initiatives to support diagnosis of affected patients
- Deliver solid value proposition to secure reimbursement & broad access

Germany

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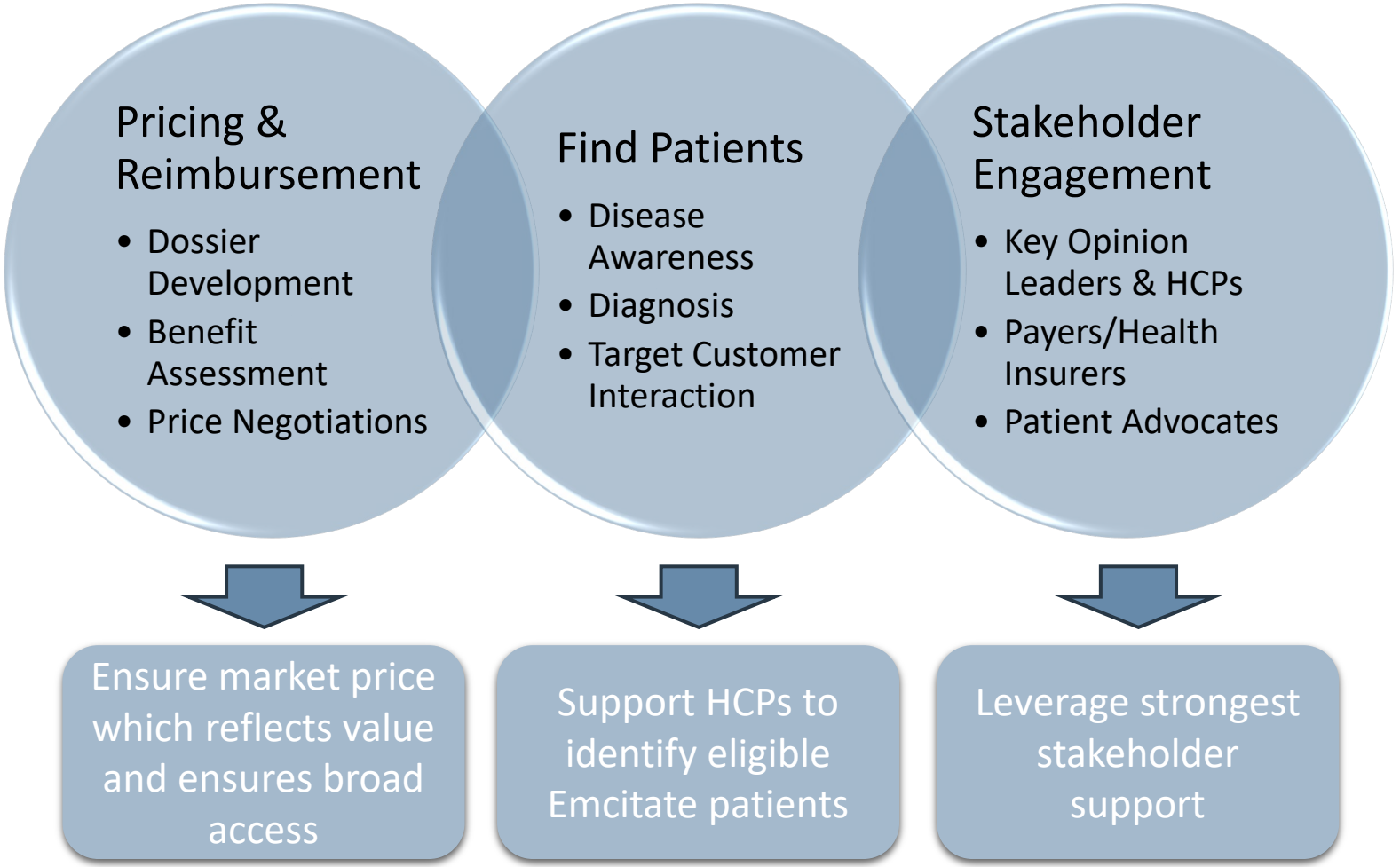
Launch Readiness Germany

Dr. med. Henna Oittinen Corbinelli, Medical Director Europe & International

Raymond Francot, General Manager for DACH, Italy and Central & Eastern Europe

Critical Elements of a successful Emcitate Launch

Ensure Access to Emcitate for all Eligible Patients upon obtaining European Marketing Authorization



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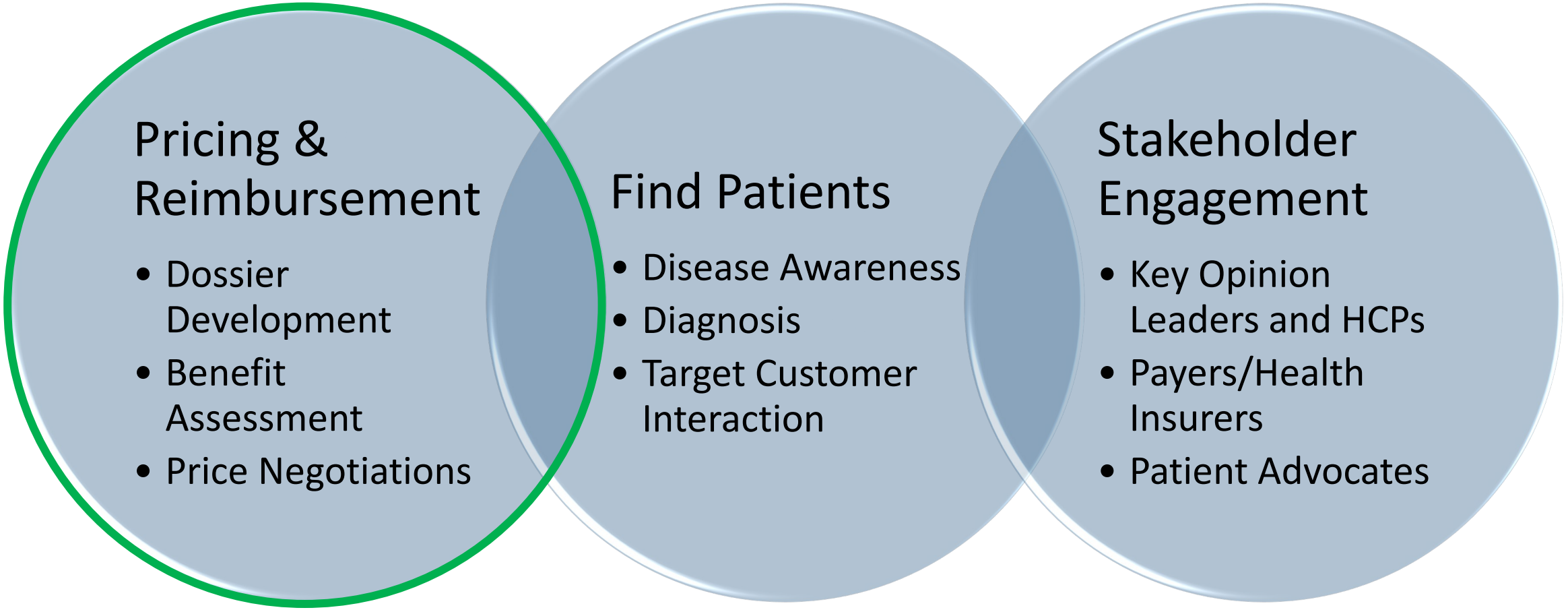
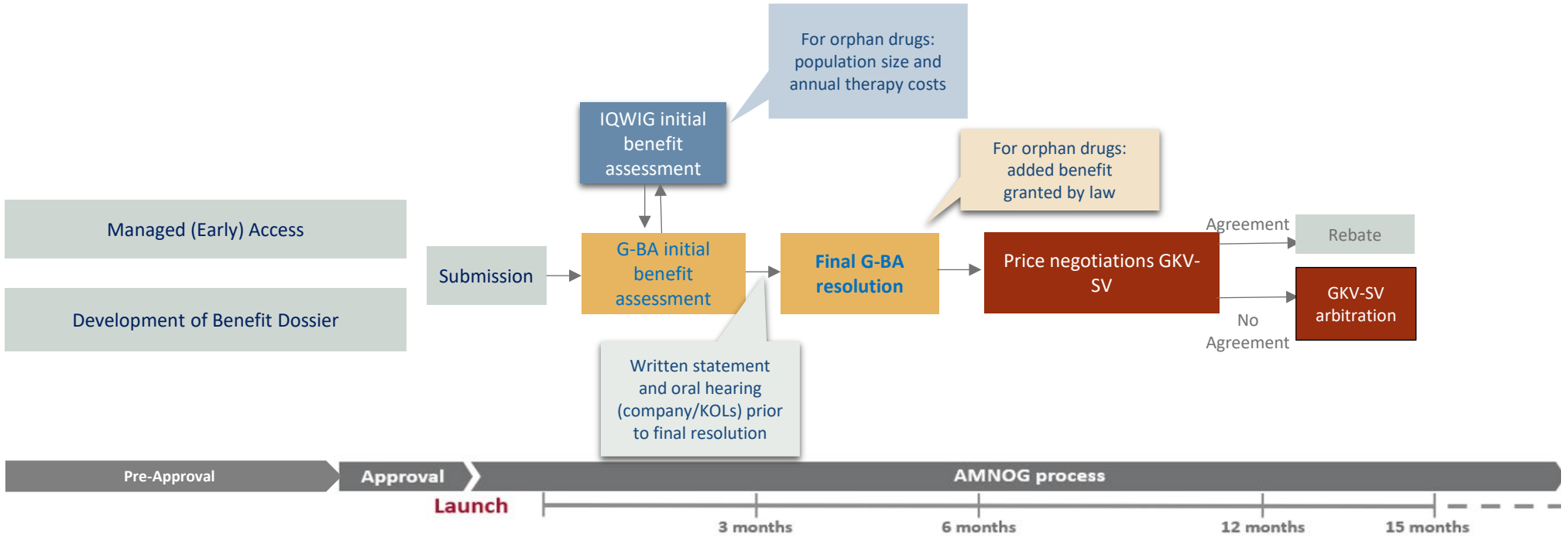


Benefit assessment and price negotiations for new drugs follow a strict and transparent process

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



4 parties involved in AMNOG process:

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Gemeinsamer Bundesausschuss

IQWiG

GKV

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

Opportunities for a successful AMNOG Process are identified

Opportunities

- **Orphan disease status** offers respective argumentations
- **High unmet medical need** for treating at all ages
- Emcitate is **already prescribed through managed access program** by renowned hospitals and KOLs have positive experience treating patients with Emcitate
- Strong **stakeholder support** is emerging (KOL, PAG)
- **No available treatment to** treat thyrotoxicosis in MCT8 deficiency
- **ETA Guidelines** support Emcitate treatment

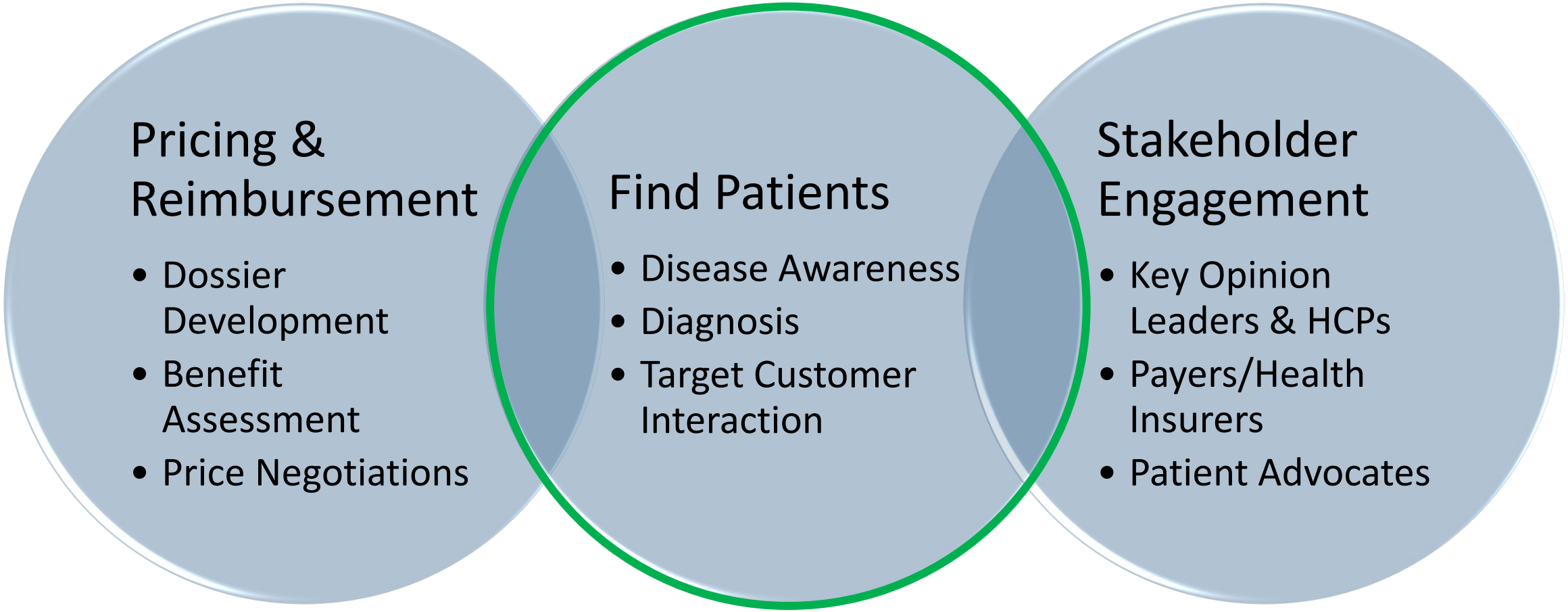
Implications

- **Implement comprehensive and sound dossier strategy supported by and aligned with EPAR/SmPC**
  - Focus on **1st treatment for ultra-rare** MCT8 deficiency, addressing a high unmet medical need
  - **Strong value story** around the meaning/burden of thyrotoxicosis in short- and long-term aspect
- **Stakeholder support** is crucial to outline necessity, unmet need and relevance of Emcitate
- Prepare and execute **negotiation strategy** to agree on price that reflects value and ensure access for eligible patients

KOLs: Key Opinion Leaders
PAG: Patient Advocacy Group
EPAR: European Public Assessment Report
SmPC: Summary of Product Characteristics

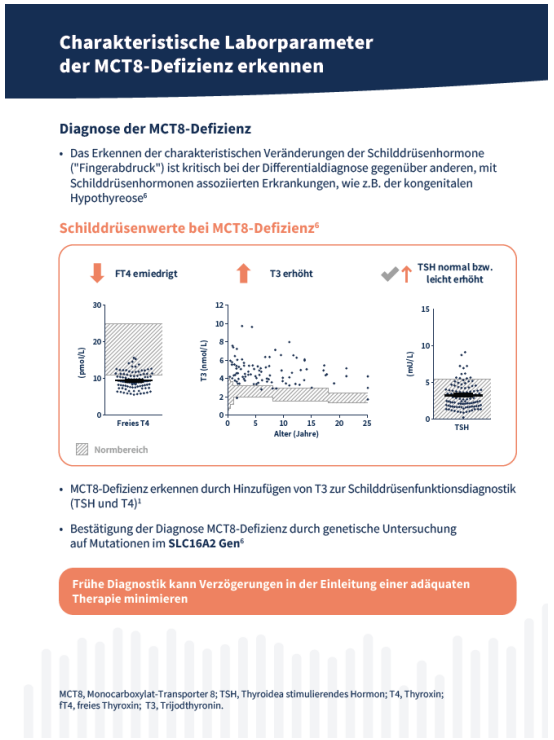
Critical Elements of a successful Emcitate Launch

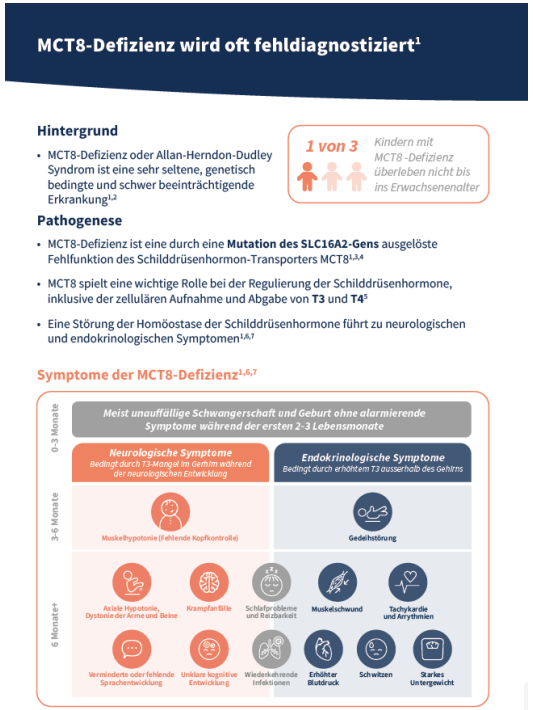
Ensure Access to Emcitate for all Eligible Patients upon obtaining European Marketing Authorization



Increasing Disease Awareness during pre-launch phase is an important success factor for successful launch of Ultra-Orphan drugs

- Importance of strengthening awareness about MCT8 deficiency as a debilitating disease with distinct concomitant clinical presentations and the importance of its early treatment
- Through disease awareness initiatives and conversations at congresses and at other occasions, we are aware of that more and more patients are diagnosed
- Through the Emcitate Managed Access Program, HCPs are able to gain first-hand experience





Critical Elements of a successful Emcitate Launch

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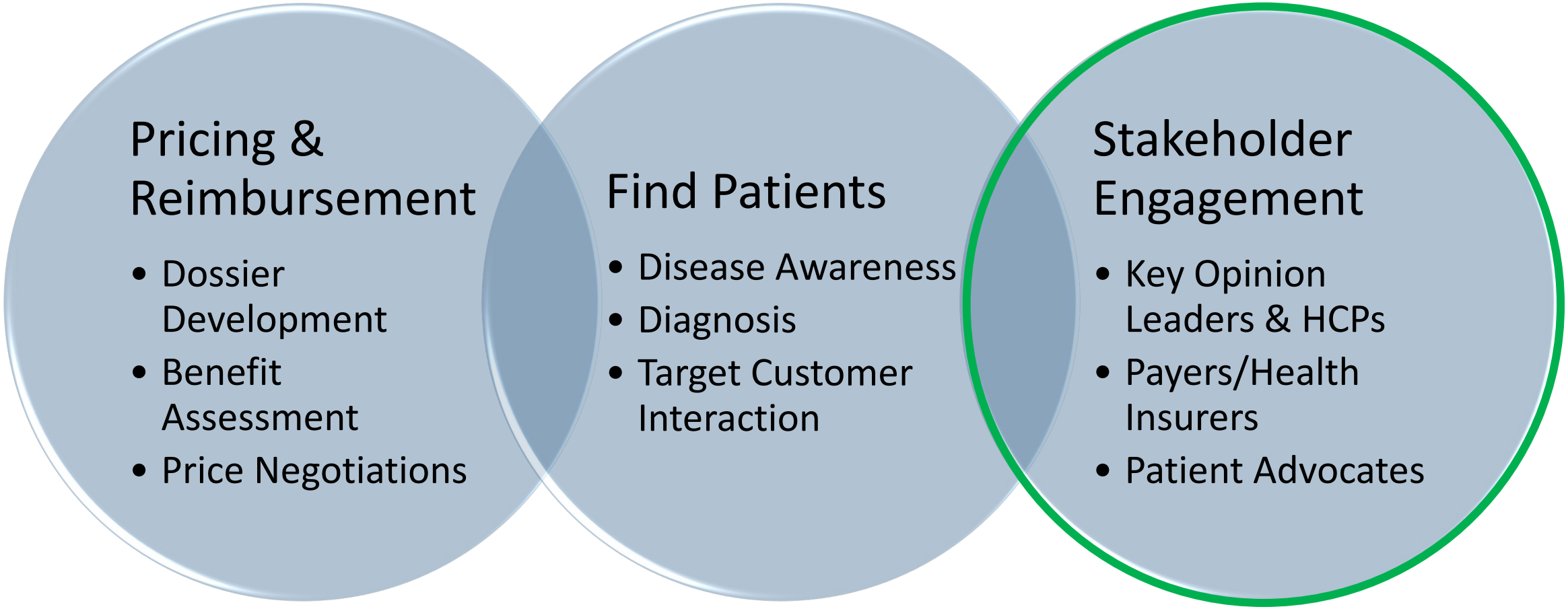


HCP Engagement Strategy

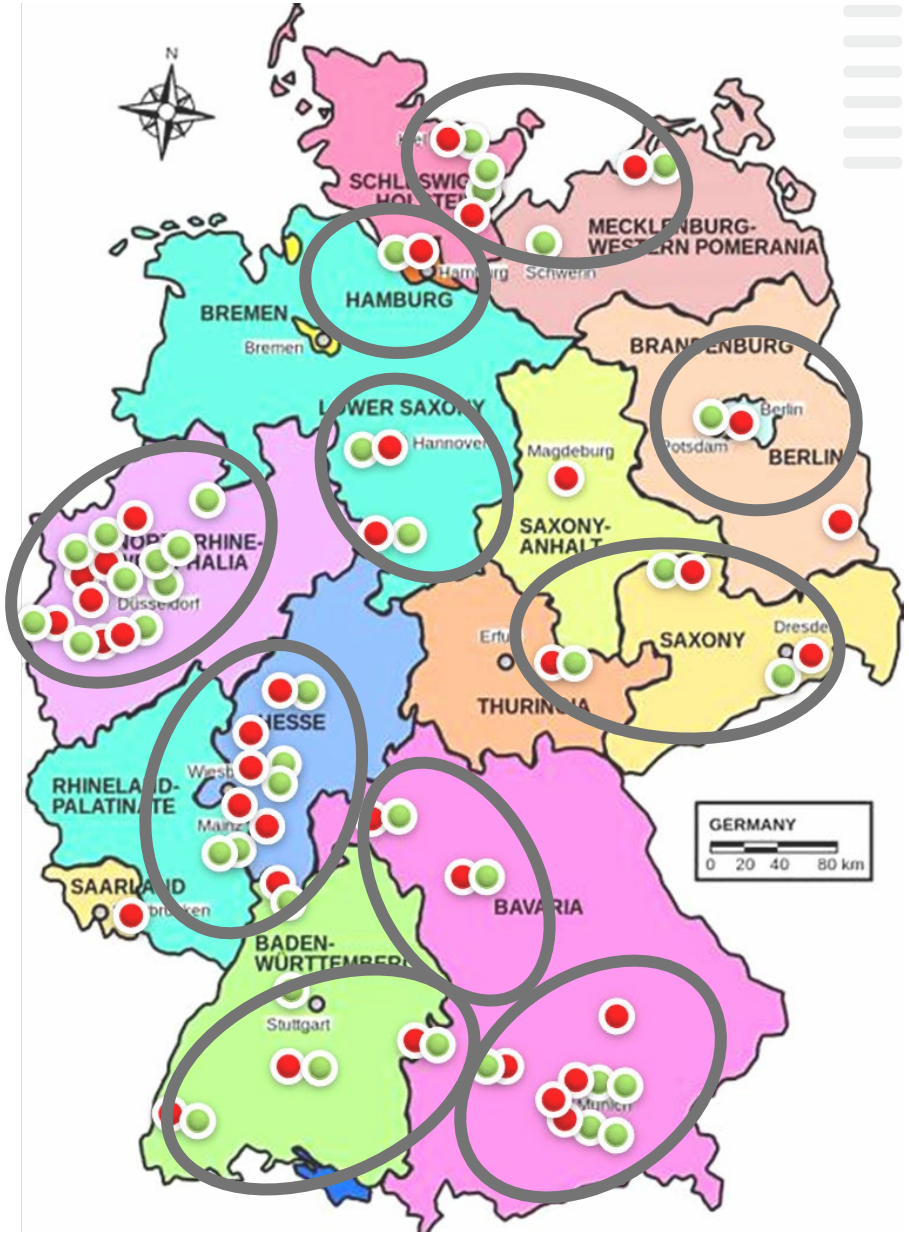
Building strong Expert base to advance management of MCT8 deficiency

MCT8 deficiency Experts

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

HCPs involved in patient journey

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



IIS: Investigator Initiated Studies
SPZs: Sozialpädiatrische Zentren – Social Pediatric Centers
ZSEs: Zentren für Seltene Erkrankungen – Centers for Rare Diseases

Strong German Expert support for increasing disease awareness

Improving patient care in MCT8 deficiency

Cross-functional core Expert group

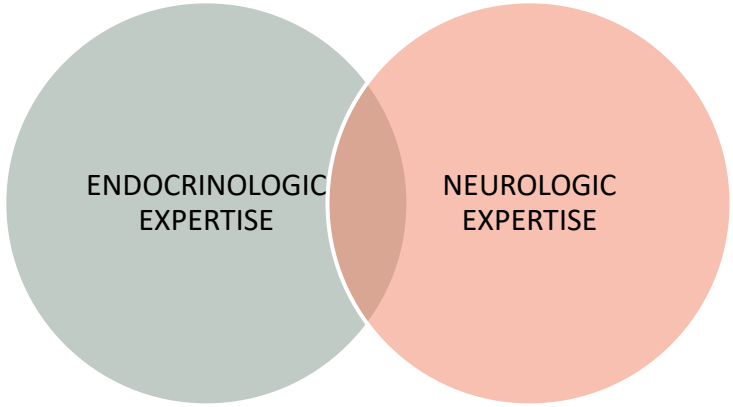
- Endocrinology/Pediatric Endocrinology
- Pediatric Neurology
- Thyroid hormone research & clinical studies
- Clinical chemistry/laboratory specialists

Main Topics 2023-2024

- Improvement of Diagnostic pathways
- Laboratory monitoring and newborn screening
- Thyrotoxicosis in MCT8 deficiency
- Cross-functional guidance on clinical monitoring

Experts agree that interdisciplinary clinical monitoring is needed to improve standard of care in MCT8 deficiency

The shared objective is to increase expertise in MCT8 deficiency by interdisciplinary exchange and collaboration of main specialities managing the patients



Awareness & collaboration is key for adequate diagnosis and treatment

Providing Access in MENAT

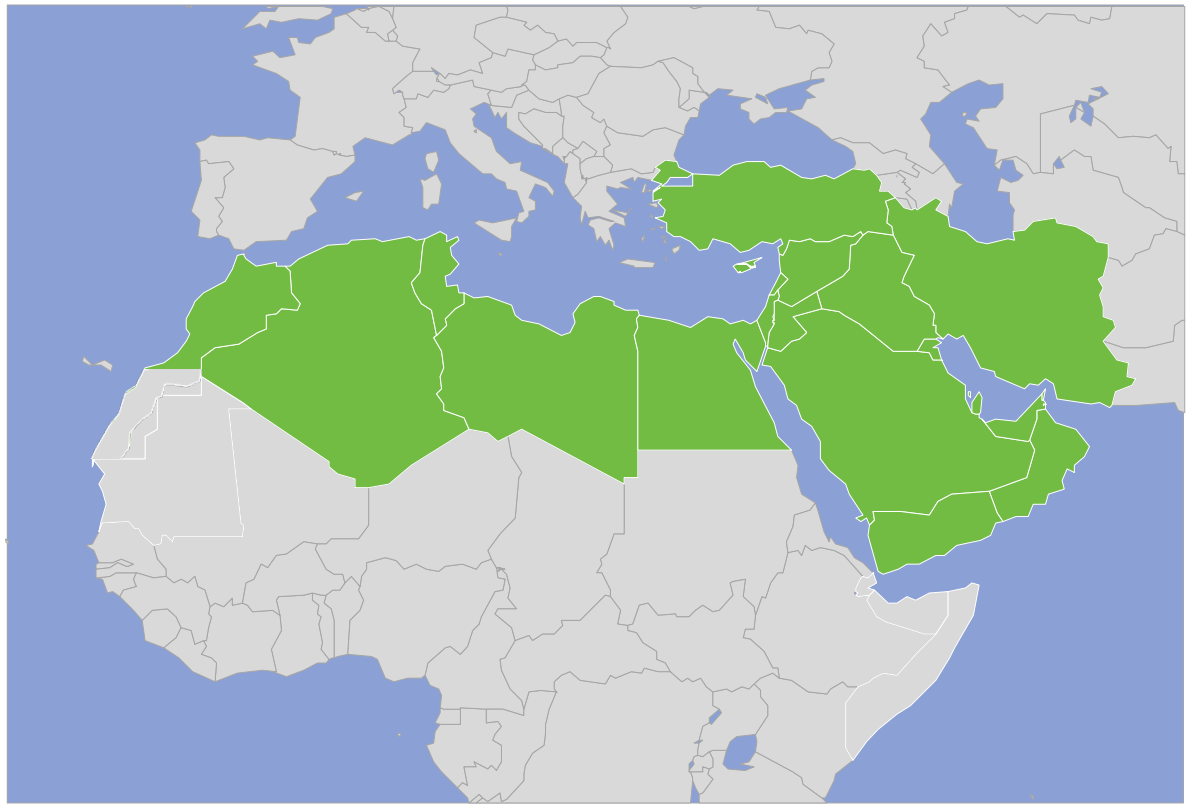
Peter Verwaijen

Global Head of Brand Strategy & Commercial Business Expansion

General Manager Benelux

The MENAT-region

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



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

Egetis' approach to the MENAT-region

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

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

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Investor Day

December 18, 2024

Q&A

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Investor Day

December 18, 2024

Break

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US regulatory pathway and the ReTRIACt study

December 18, 2024

Nicklas Westerholm, CEO

Agenda: Egetis Investor Day, December 18, 2024

Time (CET/ET)	Subject	Presenter(s)
15:00/9.00am	Welcome, CHMP opinion & corporate update	Nicklas Westerholm, CEO
15:10/9.10am	MCT8 deficiency: recent advances with tiratricol	Prof. Edward Visser, Erasmus Medical Center, NL
15:35/9.35am	Q&A	Visser & Westerholm
15:45/9.45am	Global launch preparations	Henrik Krook, Raymond Francot, Henna Oittinen-Corbinelli, Peter Verwaijen
16:20/10.20am	Q&A	Krook, Francot, Oittinen-Corbinelli, Verwaijen, Westerholm
16:30/10.30am	Break	
16:50/10.50am	US regulatory pathway & ReTRIACt study	Westerholm
17:00/11.00am	US opportunity for *Emcitate*	Anny Bedard, Ann-Marie Redmond
17:15/11.15am	Q&A	Bedard, Redmond, Westerholm
17:25/11.25am	RTH-beta and the unmet medical need	Prof. Aled Rees, Cardiff University, UK
17:50/11.50am	Q&A	Rees & Westerholm
17:55/11.55am	Concluding remarks	Mats Blom, Chairman of the Board
18:00/12.00pm	Ends	

Emcitate regulatory pathway in US

Robust data set in an ultra rare genetic disease

Triac Trial I	EMC cohort study	Natural history	Triac Trial II	Survival study	ReTRIACt Trial
N=46	N=67	N=151	N=22	N~228	N=16
• Completed 2018 (Groeneweg, 2019) • Open-label, international, multi-centre study	• 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)	• Open-label, international, multi-centre study • Focus on neurocognition, but did not meet its primary endpoints • 96 weeks safety data in young patients	• Retrospective cohort data • Comparing treated vs untreated patients on survival • Abstract at ETA by EMC	• N= 16 • Placebo controlled • Ongoing

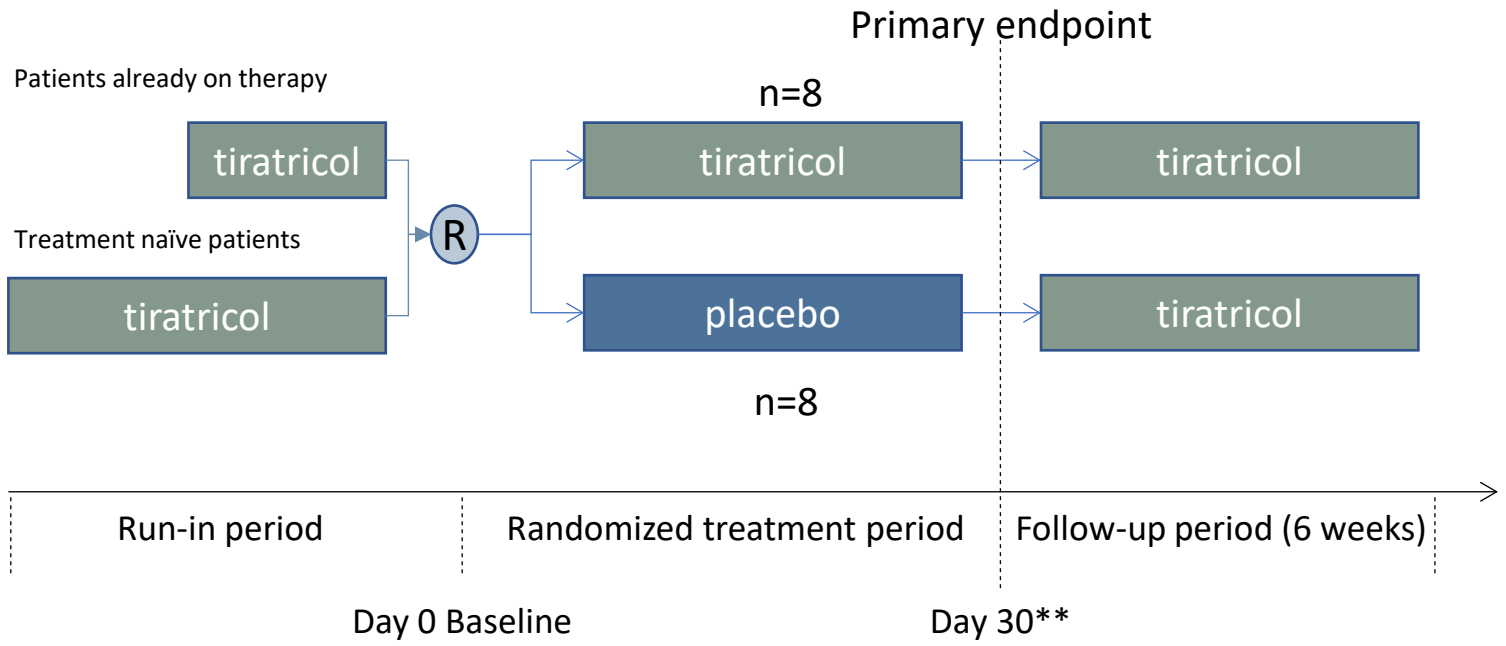
Data included in MAA

Included in D120 response

Design of the ReTRIACt clinical trial

Requested by the FDA

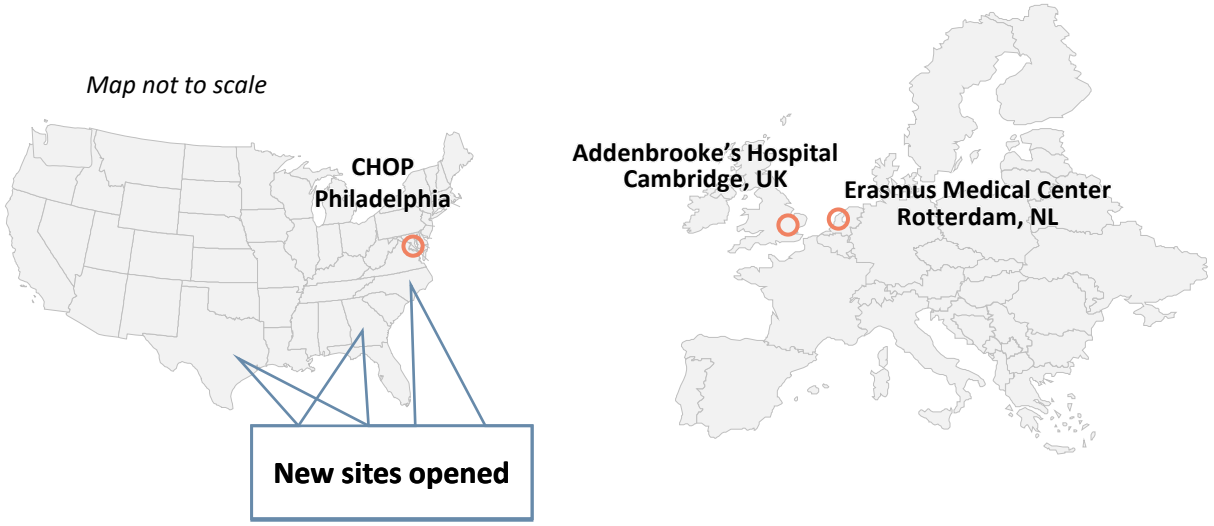
- A 30-day, randomized placebo-controlled withdrawal study in 16 patients
- Design agreed with FDA
- The study allows for inclusion of patients that are already on therapy and patients that are treatment naïve
- Treatment naïve patients require a longer run-in period to stabilize T3 levels around normal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



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

Current status of ReTRIACt trial (as of Dec. 18, 2024)



- 18 patients have been included so far, of which **8** patients have completed the randomized phase, **1** patient in the randomized phase and **4** patients are in the run-in period.
- **4** patients planned for screening in January and another 6-8 patients under evaluation for study inclusion
- 6 sites currently open, including new sites from mid 2024 in Georgia, North Carolina, Texas.
- Recruitment will continue until at least 16 patients have completed the randomized phase.

⇒ **Egetis will update the market as soon as recruitment has been completed, and subsequently when top-line results and NDA filing can be expected.**

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EGETIS THERAPEUTICS

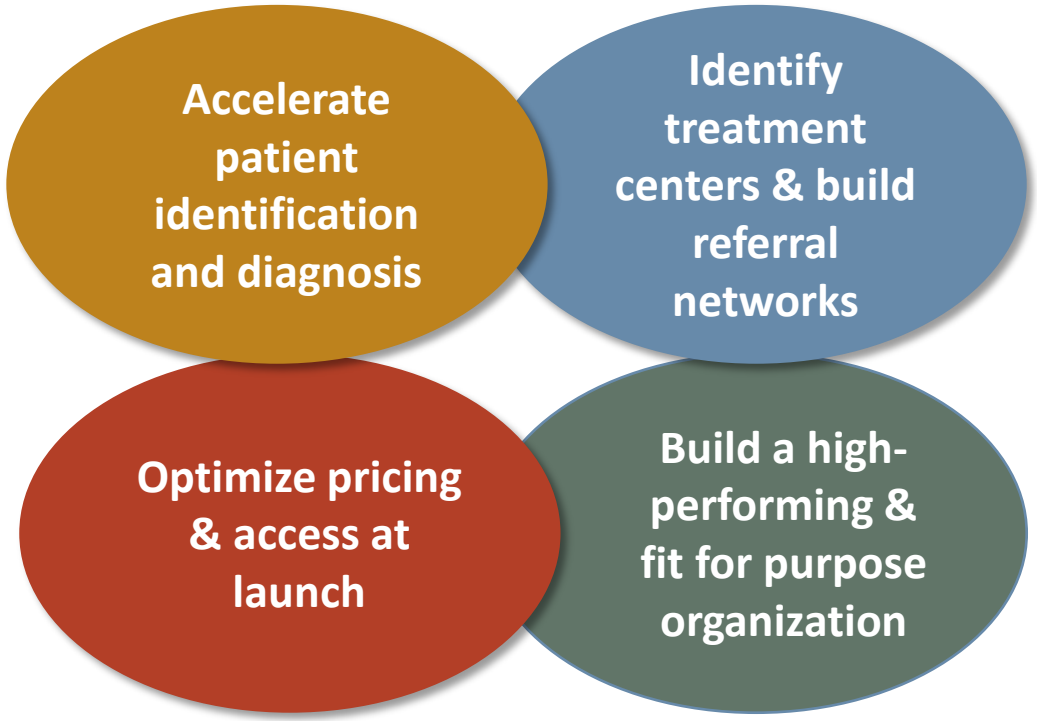
US Launch Opportunity for *Emcitate*

December 18, 2024

Anny Bedard, President North America
Ann-Marie Redmond, Head Market Access & Pricing

Progressing Strategic Priorities to Drive Launch Success

Key Drivers Shaping Egetis US Commercial Success



Key Progress Areas Driving Impactful steps towards a successful launch

- Refined our understanding of MCT8 deficiency patient journey and profile for maximizing **patient finding**
- Solidify our **access strategy** to achieve right balance between treatment cost and coverage criteria

Empowering Patient Finding Through Collaboration, Education and Innovation

Collaborations & Partnerships

- Partnering with **Advocacy Groups** to maximize impact through aligned strengths
- Engaging social **media-savvy advocates** to amplify patient outreach
- Partnering with high-impact media channels to expand disease awareness
- Collaborating with **genetic diagnostic labs** to increase disease identification

Education & Awareness

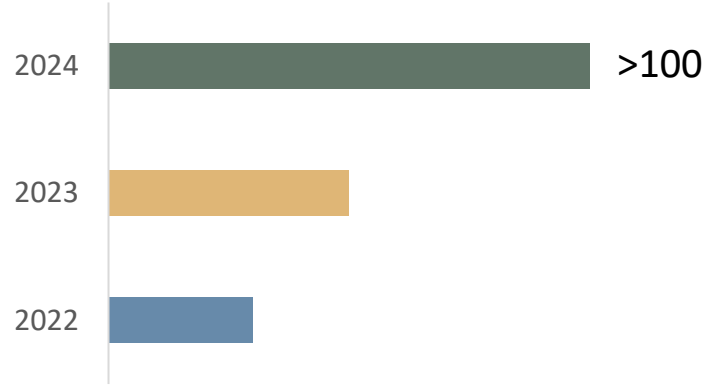
- Clinical trial recruitment
- Expanded Access Program
- Targeted conferences
- Continued Medical Education (CME) program
- Digital campaigns

Innovative Data-Driven Approach

- Leverage advanced analytics of Real-World Data and genetic test results to develop a “blueprint” of MCT8 deficiency
- Develop tools to enable identification of patients who might otherwise be missed
- Support physicians and patient Advocacy Groups to recognize MCT8 deficiency earlier

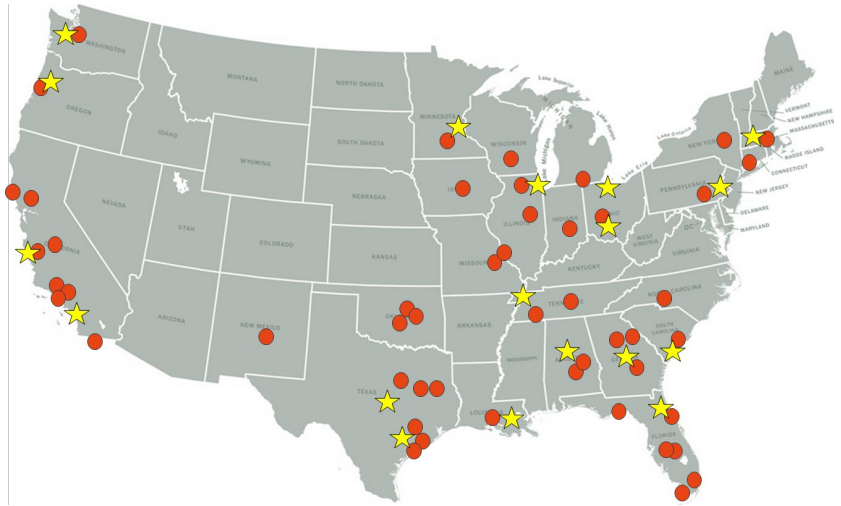
Building Momentum to Scale Patient Finding Efforts

Known confirmed diagnosed MCT8 deficiency patients



Naive or treated under early access

- **Hospitals with known MCT8 deficiency patients**
- **Tiratricol Expanded Access Program sites**

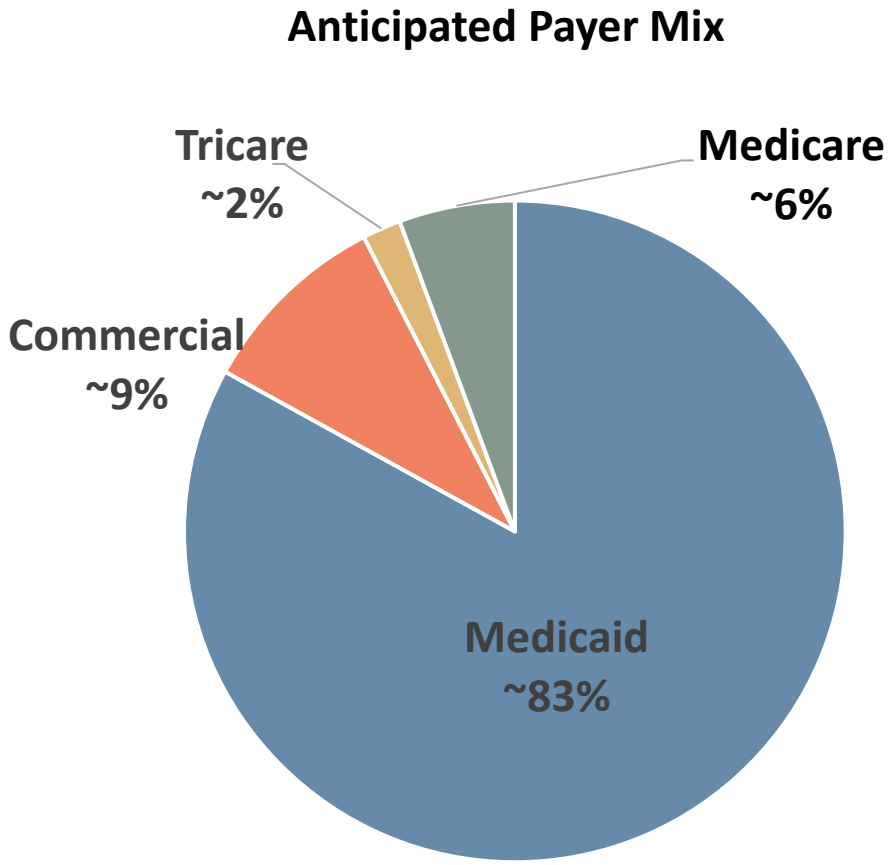


Accelerate patient finding efforts by integrating advanced data-driven insights into our existing initiatives

Establishing Strong Payer Relationships to Support Broad Access Across Priority Segments

Medicaid and Commercial

Early engagement to raise awareness and education of MCT8 deficiency to support speed to coverage and reimbursement upon approval



Source: Real world data analysis 2024/Accenture

Balancing Annual Treatment Costs and Broad Access

Analogues

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

Access

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

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

Prioritizing Impactful Activities to Optimize Access

Early Engagement

- **Foster Key Opinion Leader (KOL) champions** with strong understanding of MCT8 deficiency
- **Payer education** on MCT8 deficiency
- **Engagement strategy** (i.e., who, what messaging, when)

Seamless Patient Provider Experience

- **Operationalize fit for purpose provider patient experience model**
- **Design of support services to navigate Prior Authorization** for providers and patients
- **Patient assistance programs** for out-of-pocket cost concerns

Importance of Normalizing T3

- **Payer value proposition** and objection handler
- **Real world evidence** to support documentation of benefit outside of clinical trial setting

Building Our Team, Capabilities and Infrastructure in Stepwise Approach for a Successful US Launch

Supply Chain
- *3PL*
- *Specialty Pharmacy*

Market Access
- *Patient Services*
- *Payer Engagement*

Medical Affairs
- *Medical Scientific Liaisons*
- *Medical Info*

Marketing
- *Brand Manager*

Commercial Operations
- *Business Insights & Analytics*
- *CRM*

3PL: Third party logistics

Delivering Impact at Launch

Critical Priorities 2025

- ReTRIACt results and NDA submission
- Patient readiness for treatment at launch
- Strong HCP commitment
- Committed and active patient advocacy community
- Maximized access
- Purposed built organization

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Investor Day

December 18, 2024

Q&A

Resistance to Thyroid Hormone β and the Unmet Medical need

Professor Aled Rees

Consultant Endocrinologist

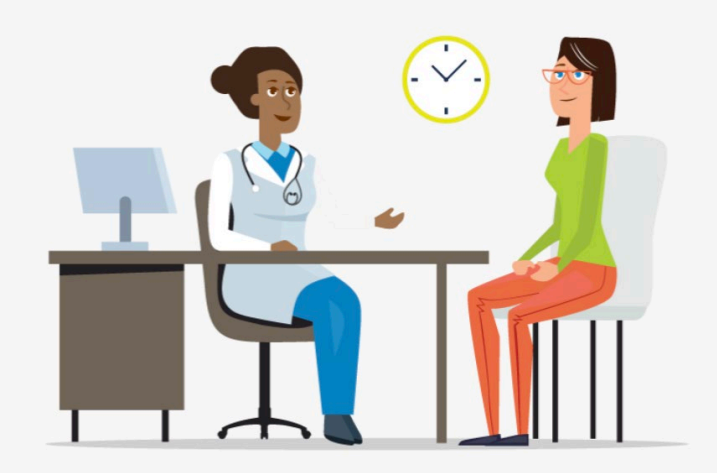
Cardiff University

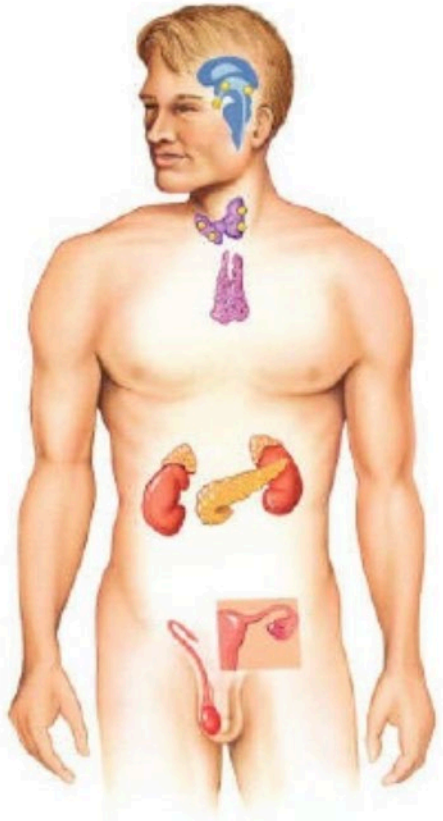
United Kingdom

18th December 2024

Resistance to Thyroid Hormone BETA
=
RTHβ

Endocrinology





Understand how hormones work – in health and disease

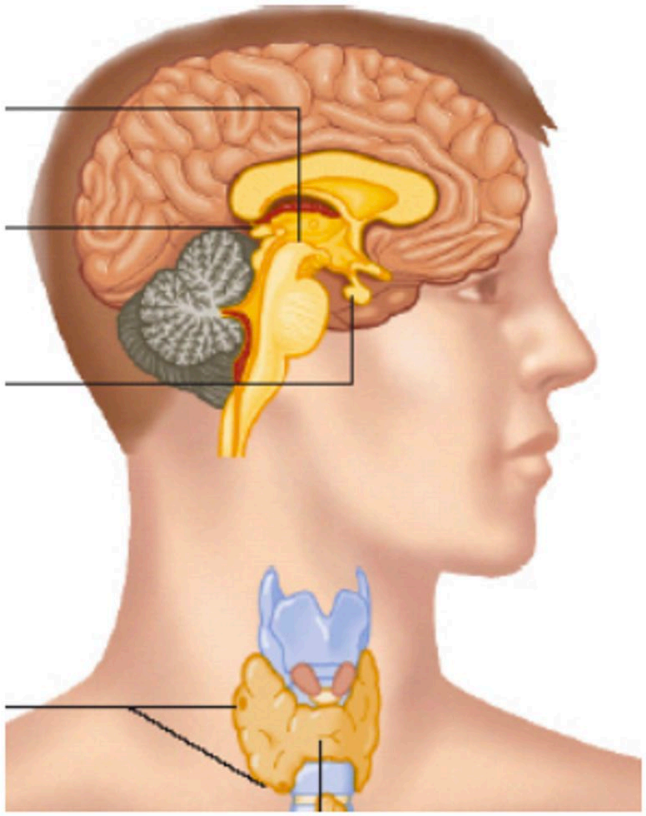
Overview

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)

+

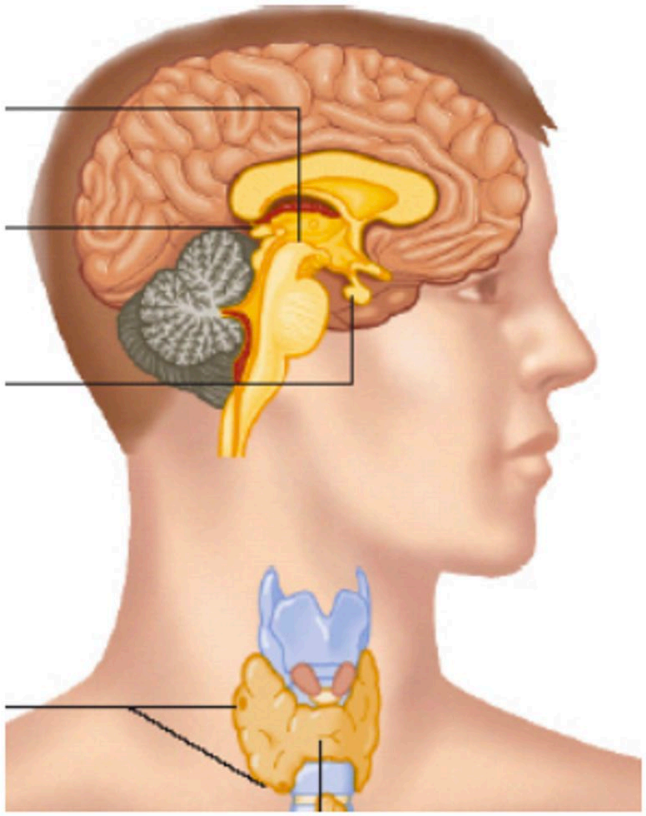
Thyroid Hormones
T4 and T3

Thyroid Hormone Production: “The Feedback Loop”

Pituitary

Thyroid

-

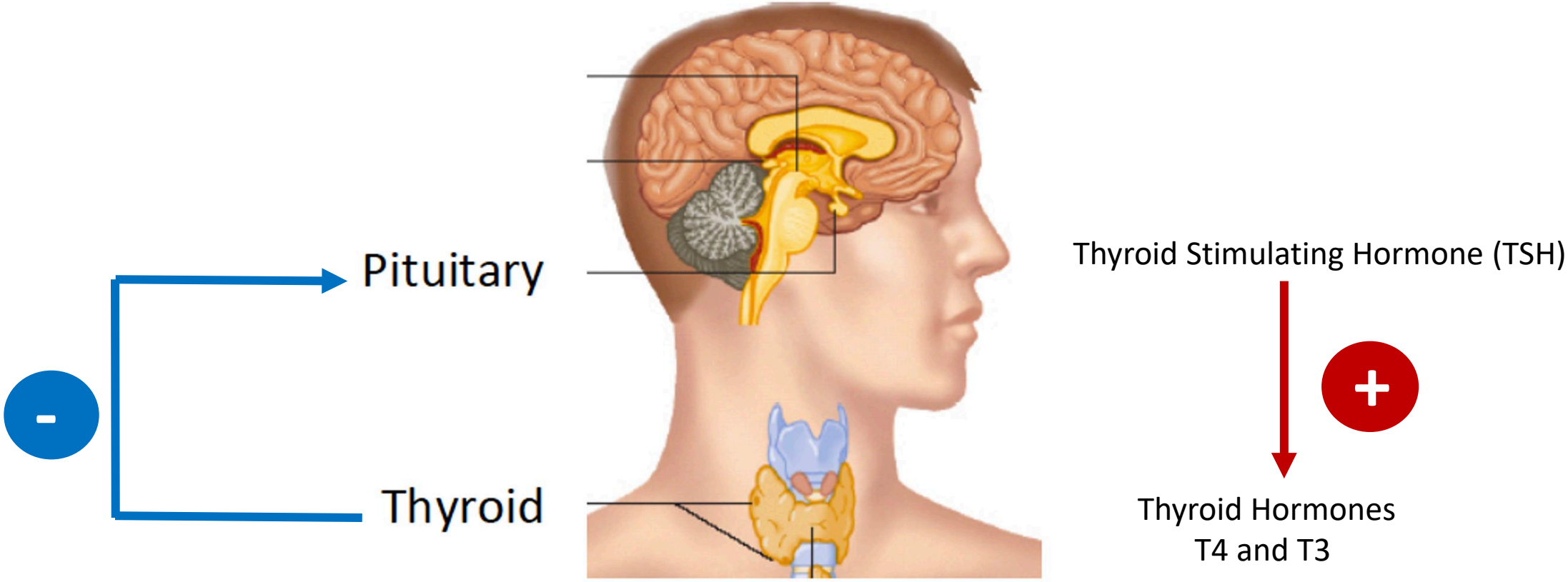


Thyroid Stimulating Hormone (TSH)

+

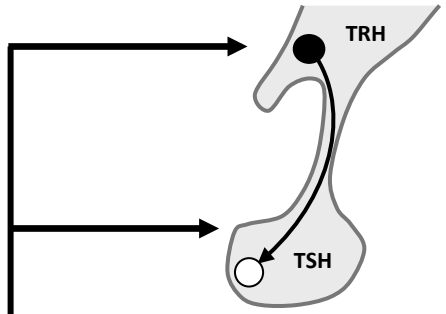
Thyroid Hormones
T4 and T3

Thyroid Hormone Production: “The Feedback Loop” Example

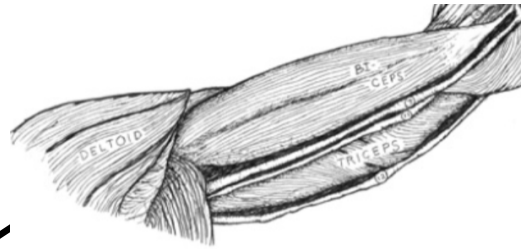


	Overactive Thyroid Gland	Example levels	Normal Levels
TSH	LOW	<0.03	0.27-4.2
T4	HIGH	45	12-22
T3	HIGH	22	3.1-6.8

Normal Thyroid Hormone action

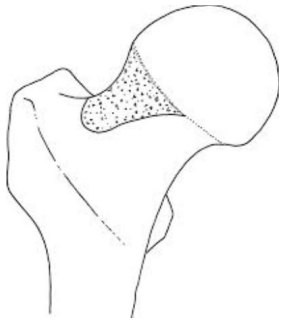


T3 T4



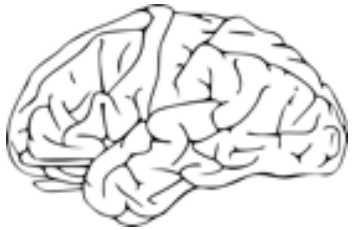
Muscle

Metabolic Rate



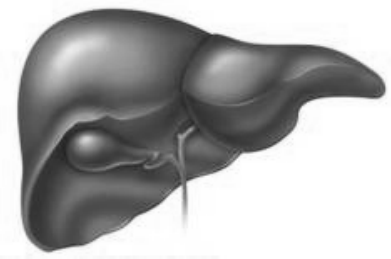
Bone

Bone maturation
Bone turnover
Growth



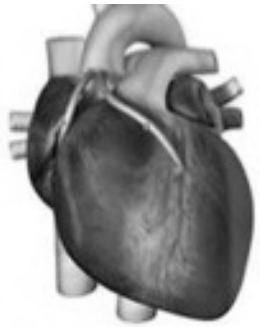
CNS

Brain development
Myelination



Liver

SHBG production
Cholesterol metabolism



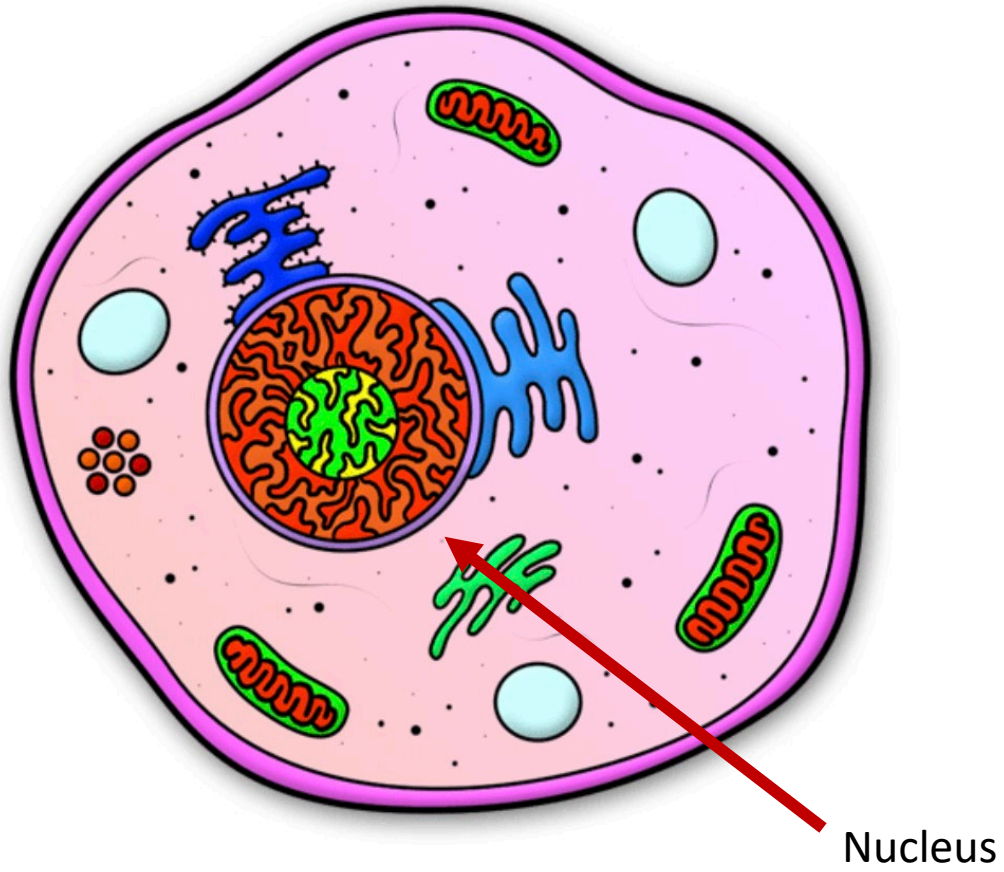
Heart

Inotropic effects
Chronotropic effects

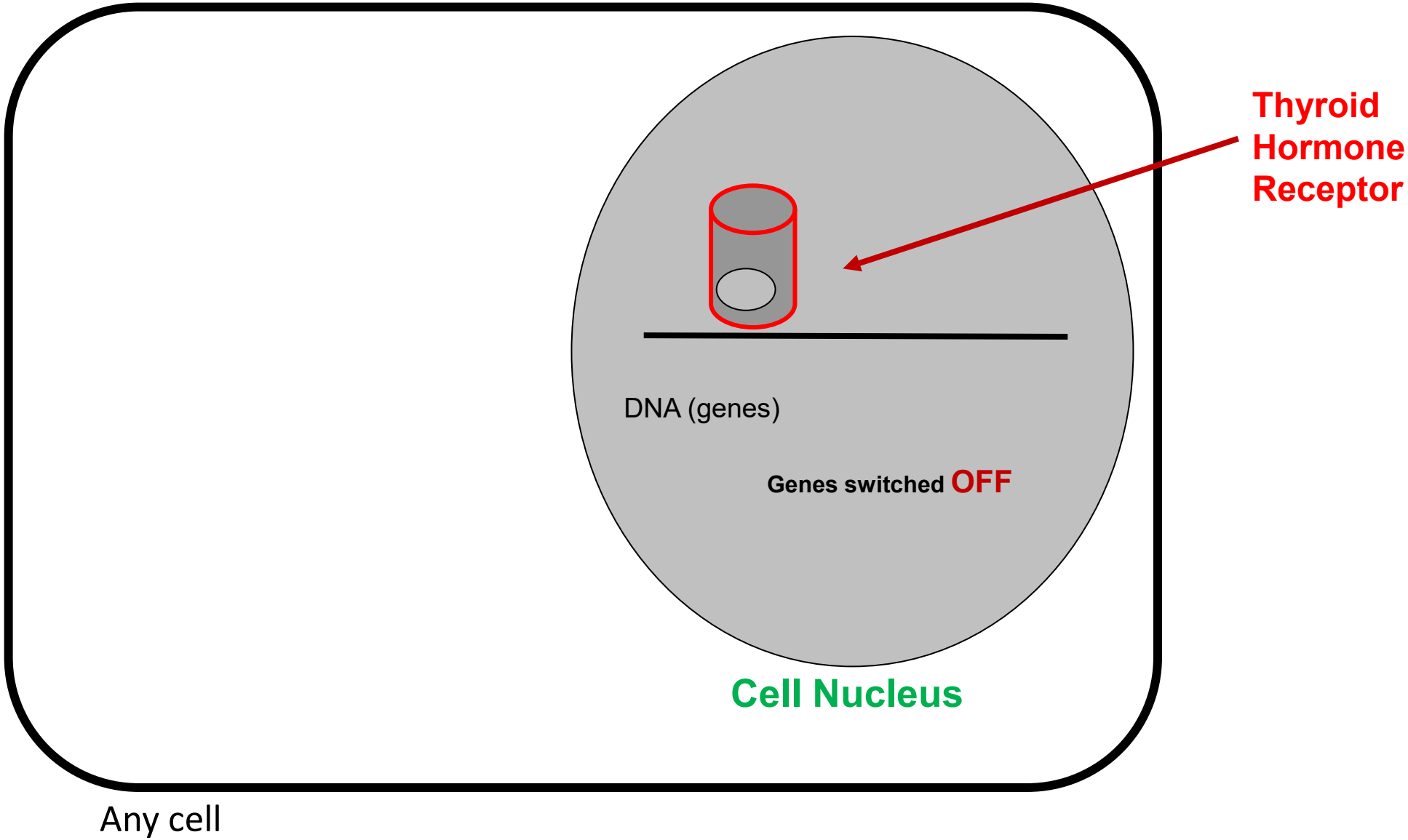
Adapted from Moran C. *Best Pract Res Clin Endocrinol Metab.* 2015. PMID 26303090

But how do Thyroid Hormones actually work?

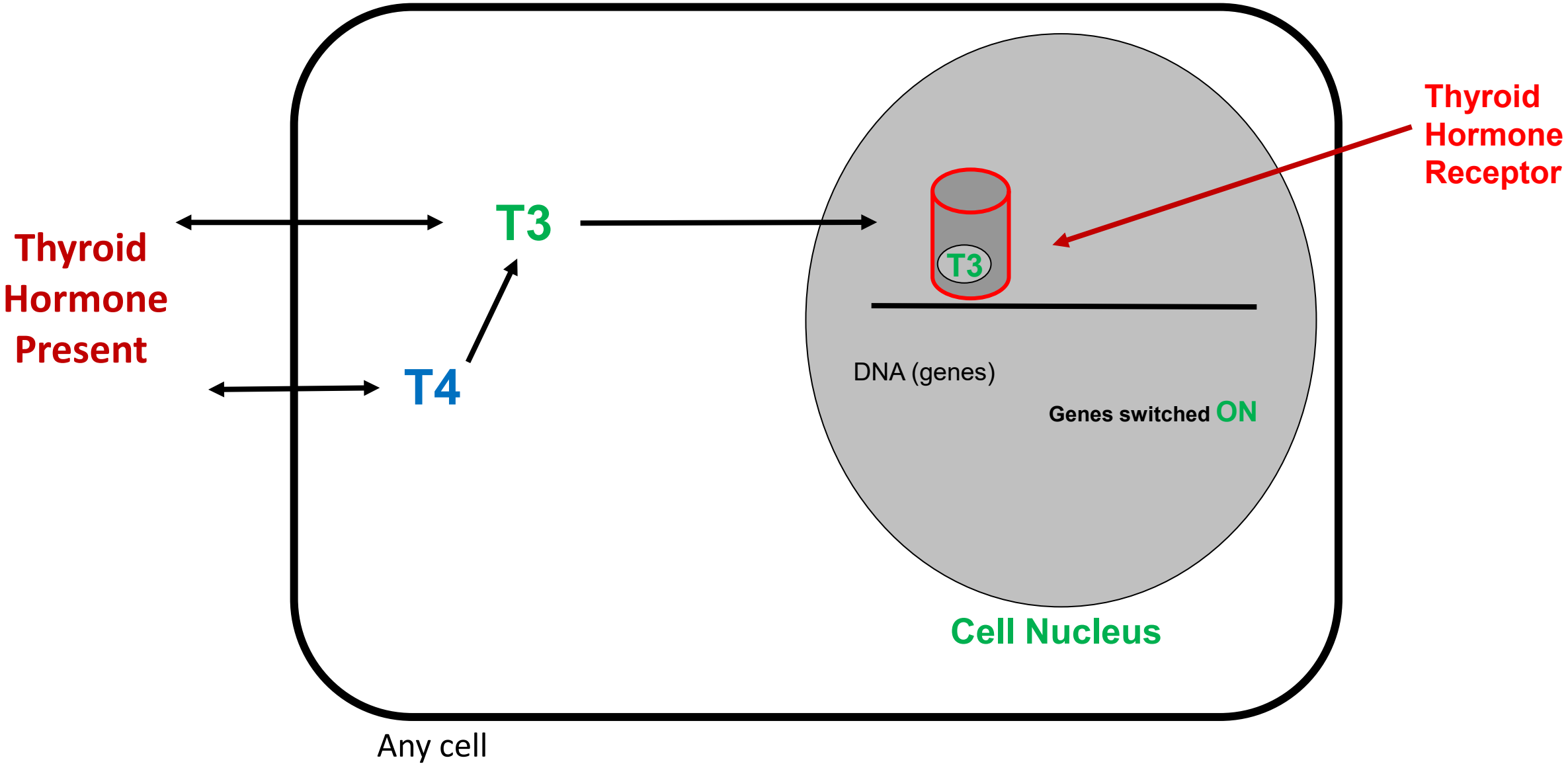
To answer this we need to go inside the cell



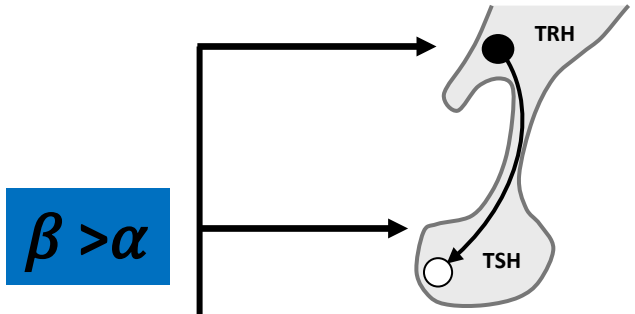
But how do Thyroid Hormones actually work?

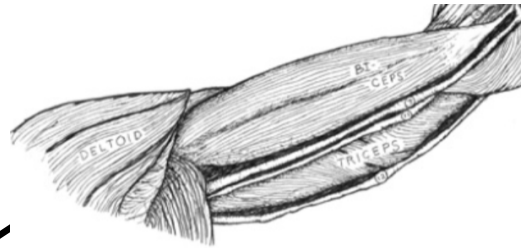


But how do Thyroid Hormones actually work?



Thyroid Hormone Receptors – TWO FORMS

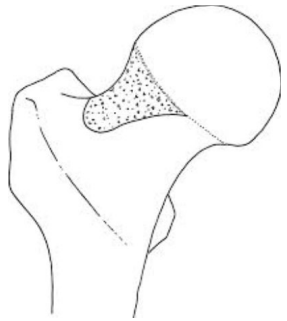




α>β

Muscle

Metabolic Rate

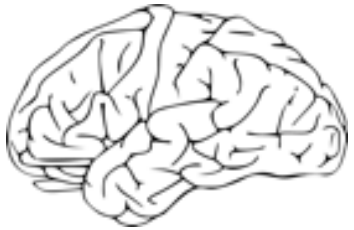


α>β

Bone

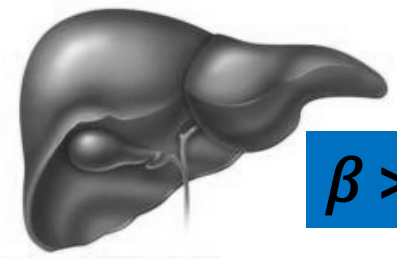
Bone maturation
Bone turnover
Growth

α>β



CNS

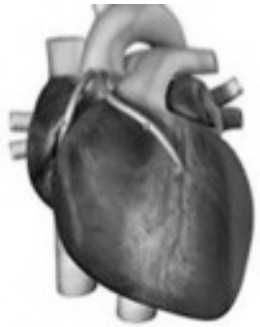
Brain development
Myelination



β >α

Liver

SHBG production
Cholesterol metabolism



α>β

Heart

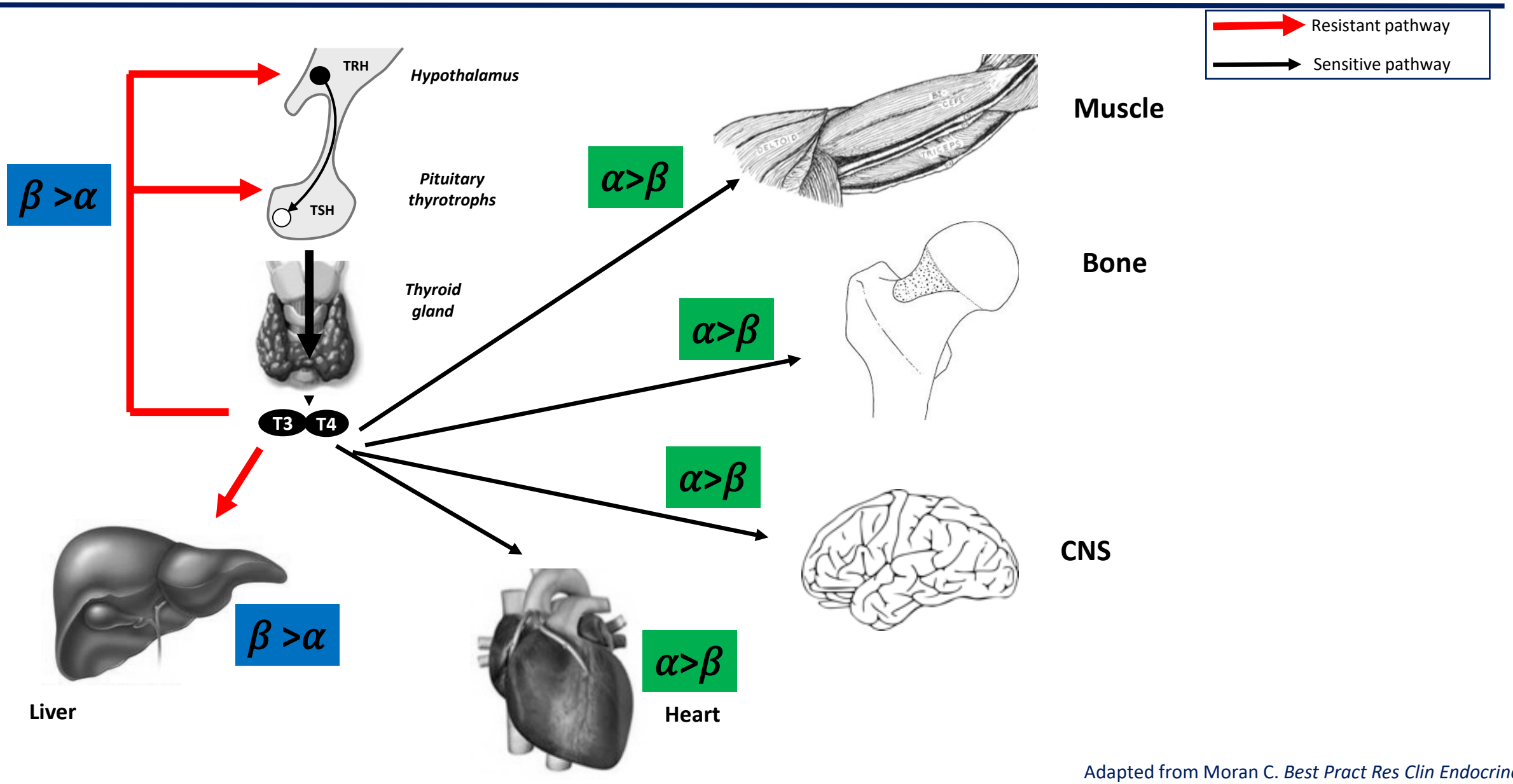
Inotropic effects
Chronotropic effects

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

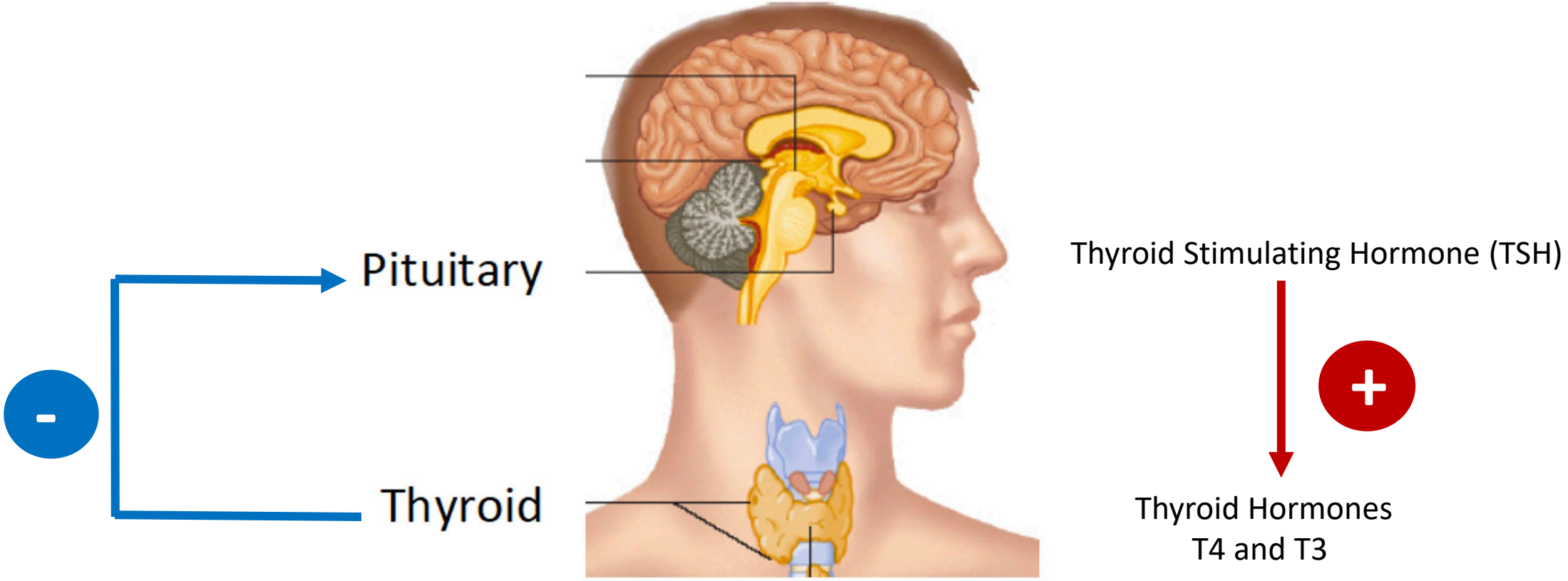


Adapted from Moran C. *Best Pract Res Clin Endocrinol Metab.* 2015. PMID 26303090

Overview

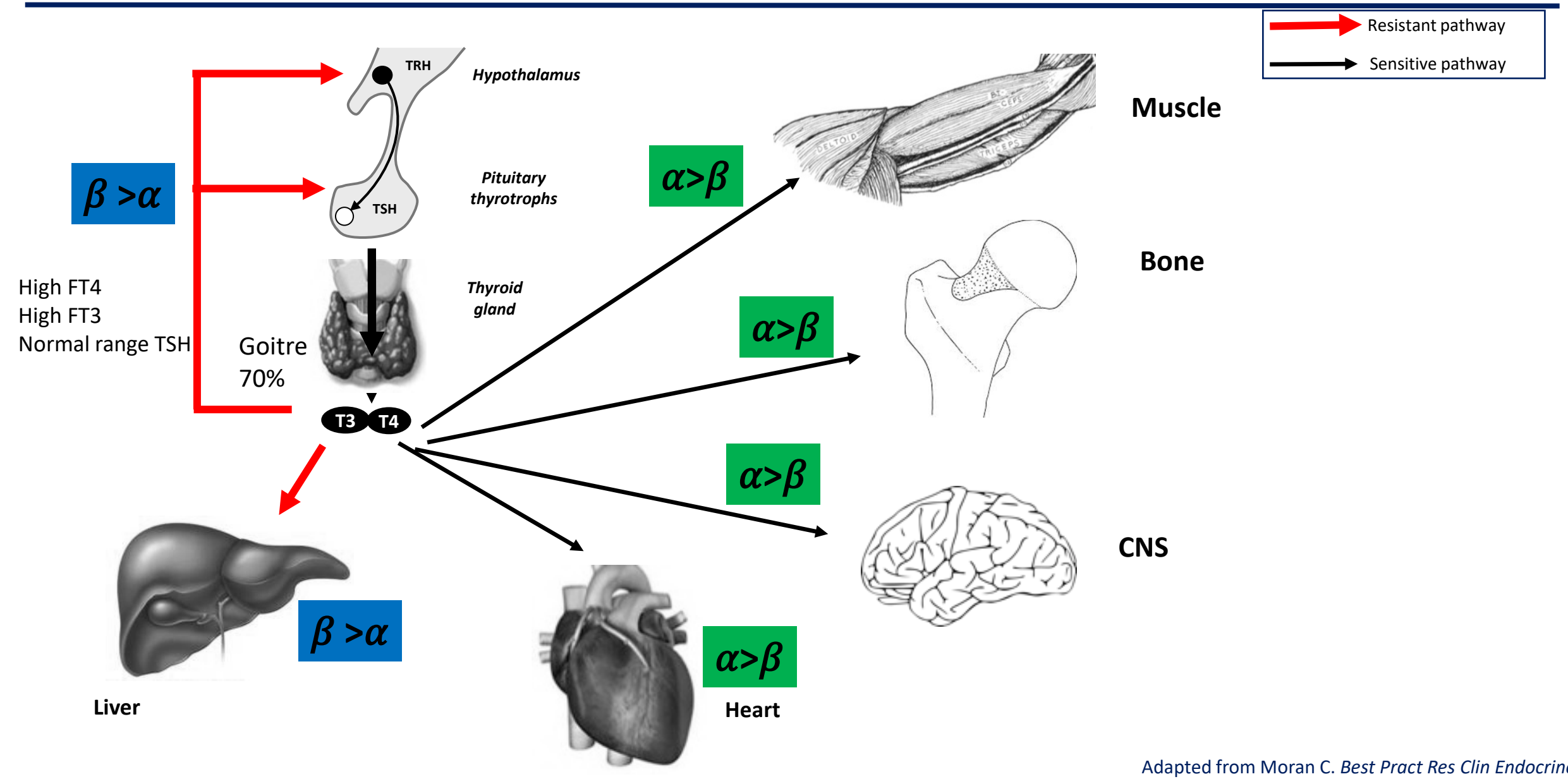
3.How is Resistance to Thyroid Hormone β diagnosed?

“The Feedback Loop” in RTHβ



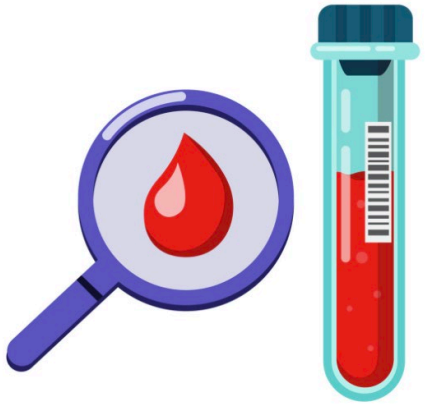
		Example levels	Normal Levels
TSH	NORMAL RANGE	4.0	0.27-4.2
T4	HIGH	45	12-22
T3	HIGH	22	3.1-6.8

RTHβ: Abnormal Thyroid blood tests

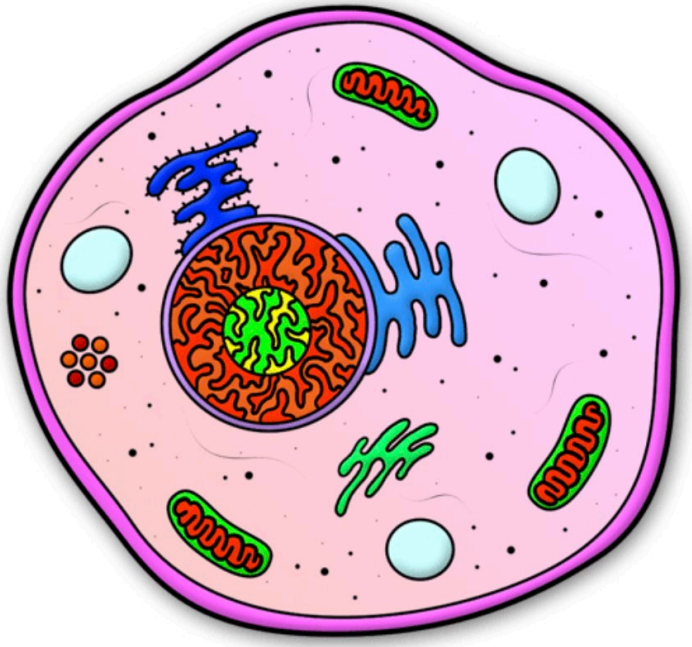


Adapted from Moran C. *Best Pract Res Clin Endocrinol Metab.* 2015. PMID 26303090

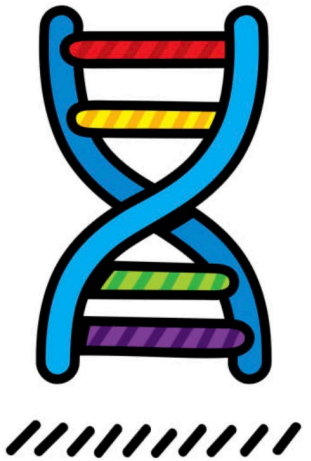
DNA testing for RTHβ



Blood test



Cell



DNA

“THRB”

Gene

Inheritance of RTHβ

Unusually inherited in an autosomal dominant pattern

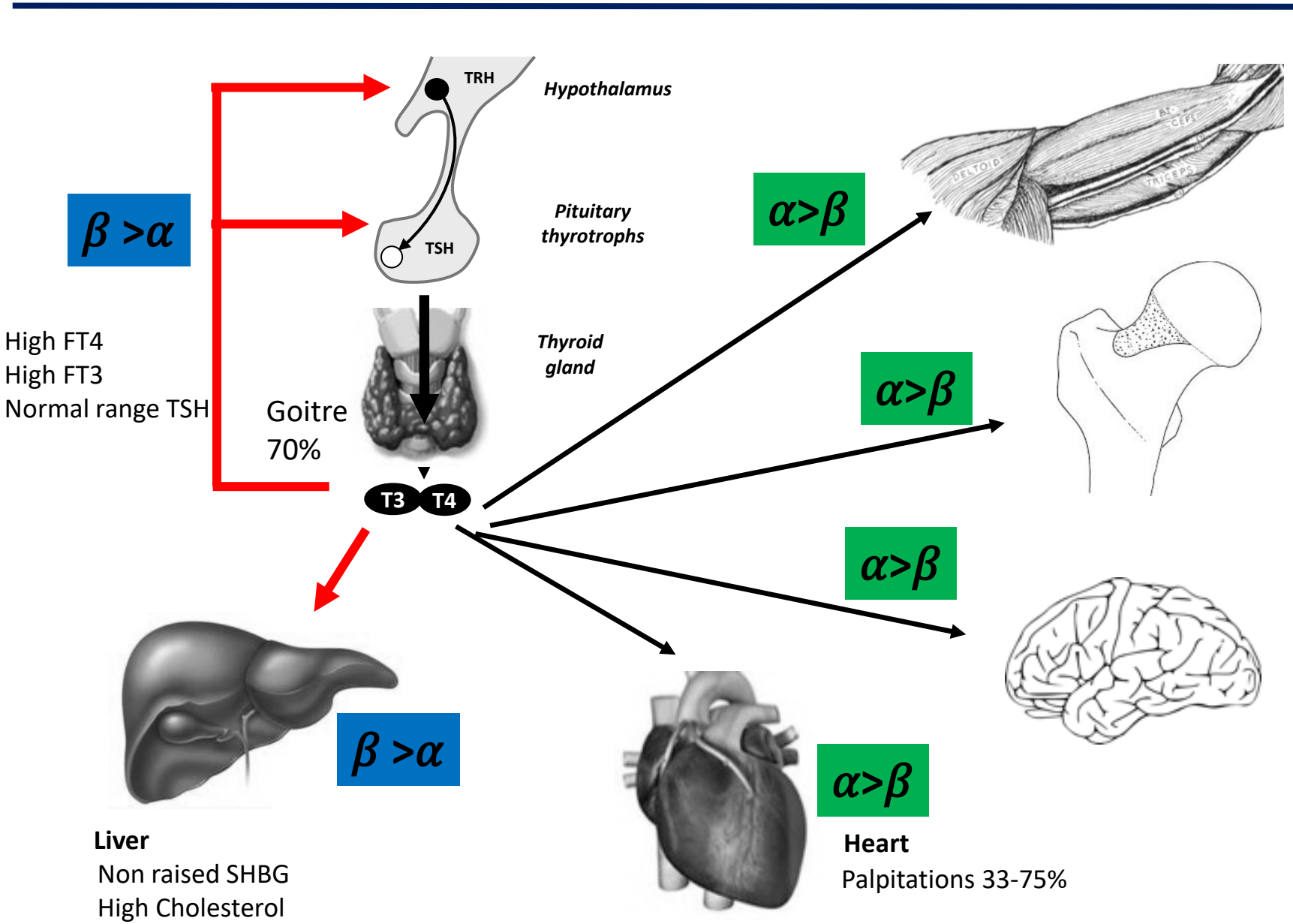
So for a father/mother with RTHβ, each of their children has a 1 in 2 chance of having the condition

1 in 20,000 to 40,000
Males = Females
Can be diagnosed at any age
Can cause many symptoms
Some people have no symptoms
Symptoms can vary over time

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%

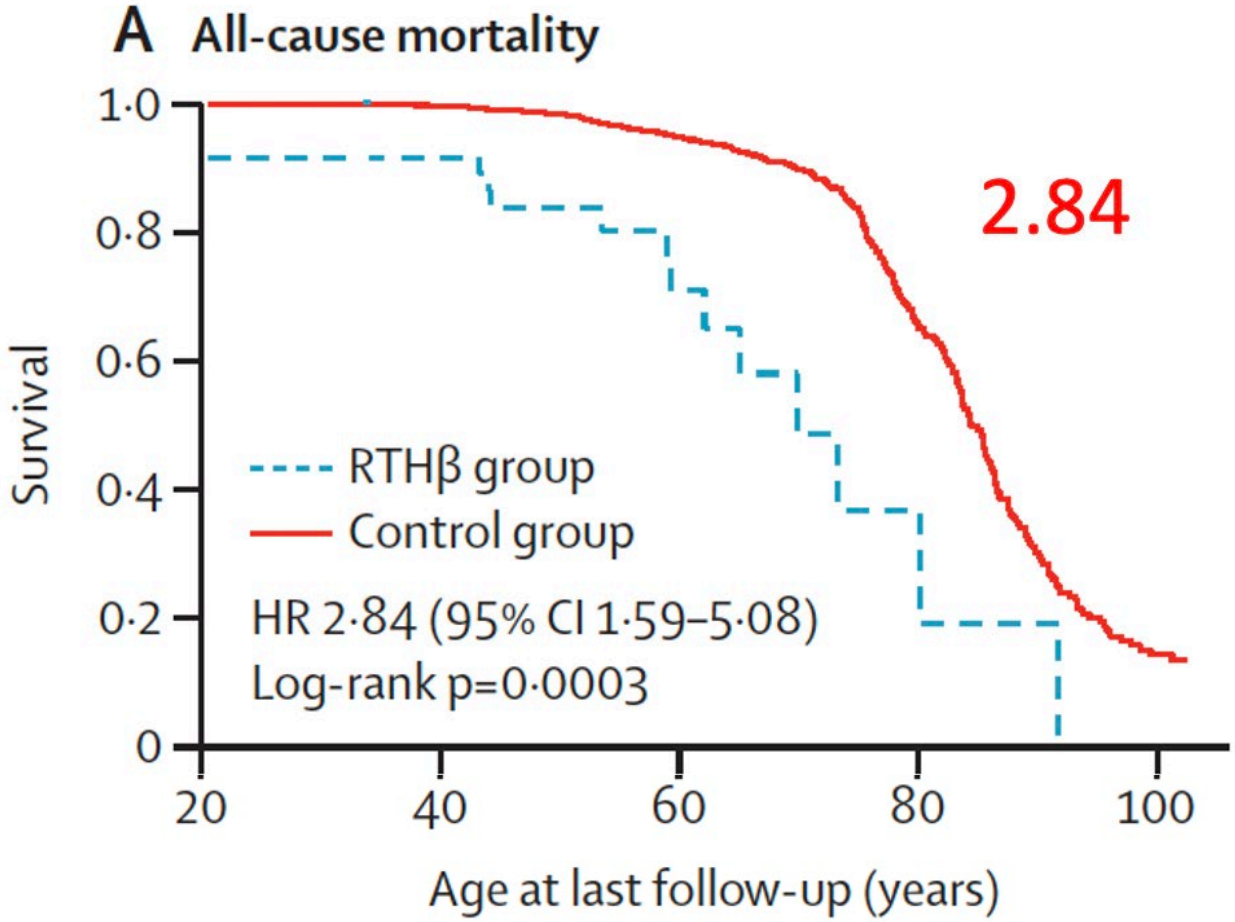
CNS
ADHD 40-60%
Poor attention, concentration
Reduced IQ 30%
Anxiety
Hyperkinetic behaviour 33-68%

Liver
Non raised SHBG
High Cholesterol
Insulin Resistance

Heart
Palpitations 33-75%

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

Increased Mortality RTHβ

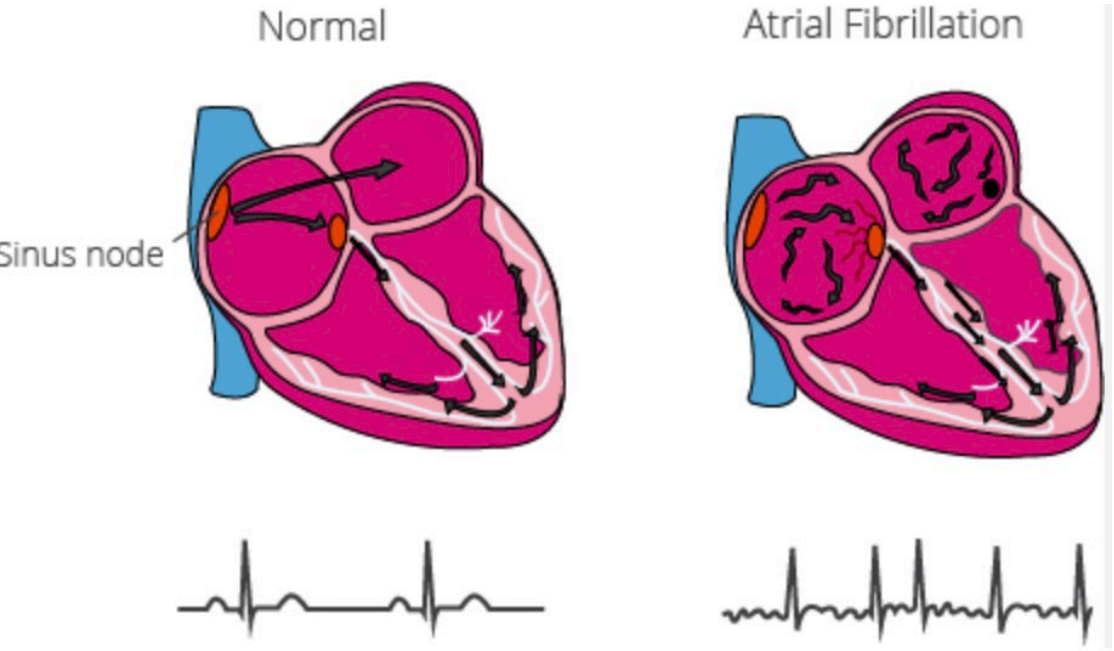


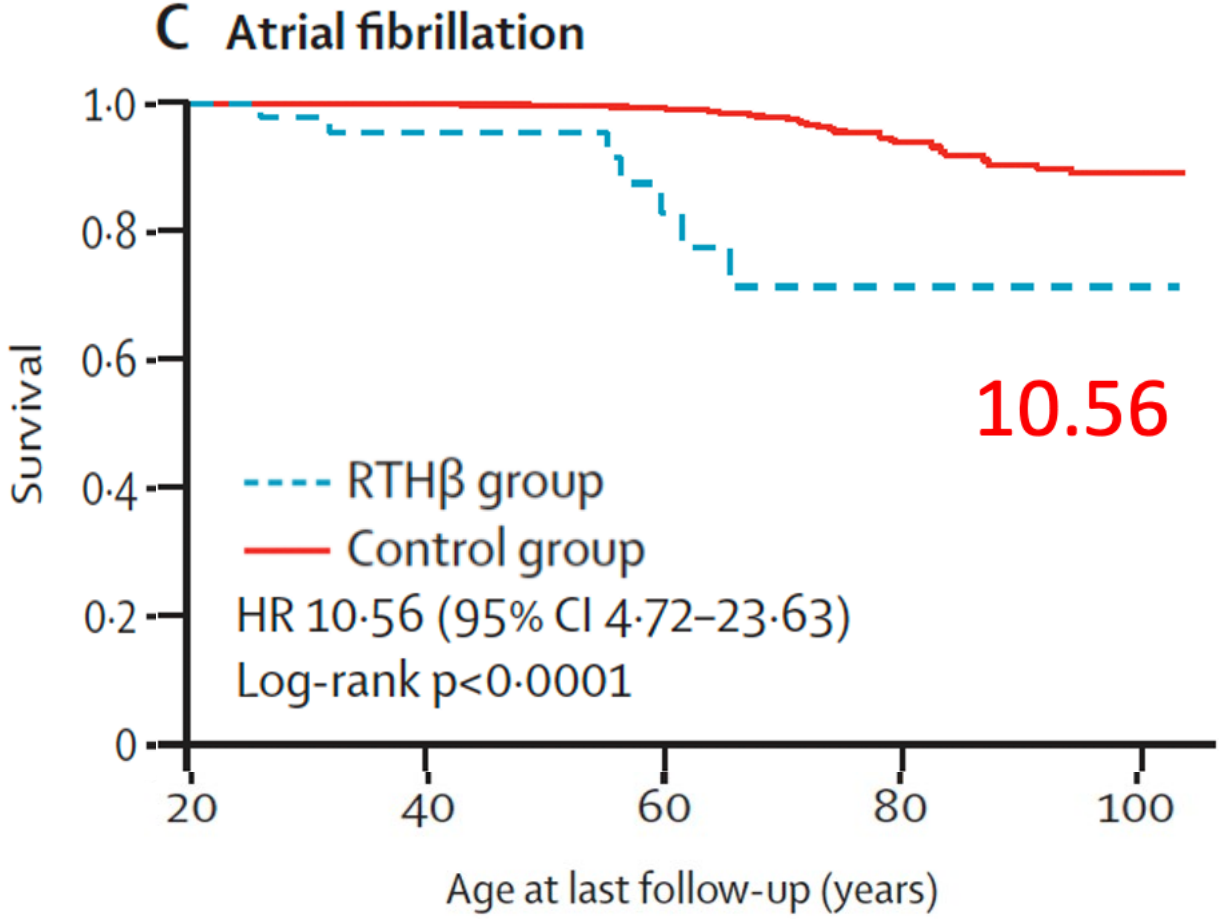
Welsh cohort
55 patients RTH Beta
2750 Age and sex matched controls
Median age 1st event 56 vs 67

Okosieme, Lancet Diabetes Endocrinology 2023. PMID 37475119

Features of RTHβ: focus on heart

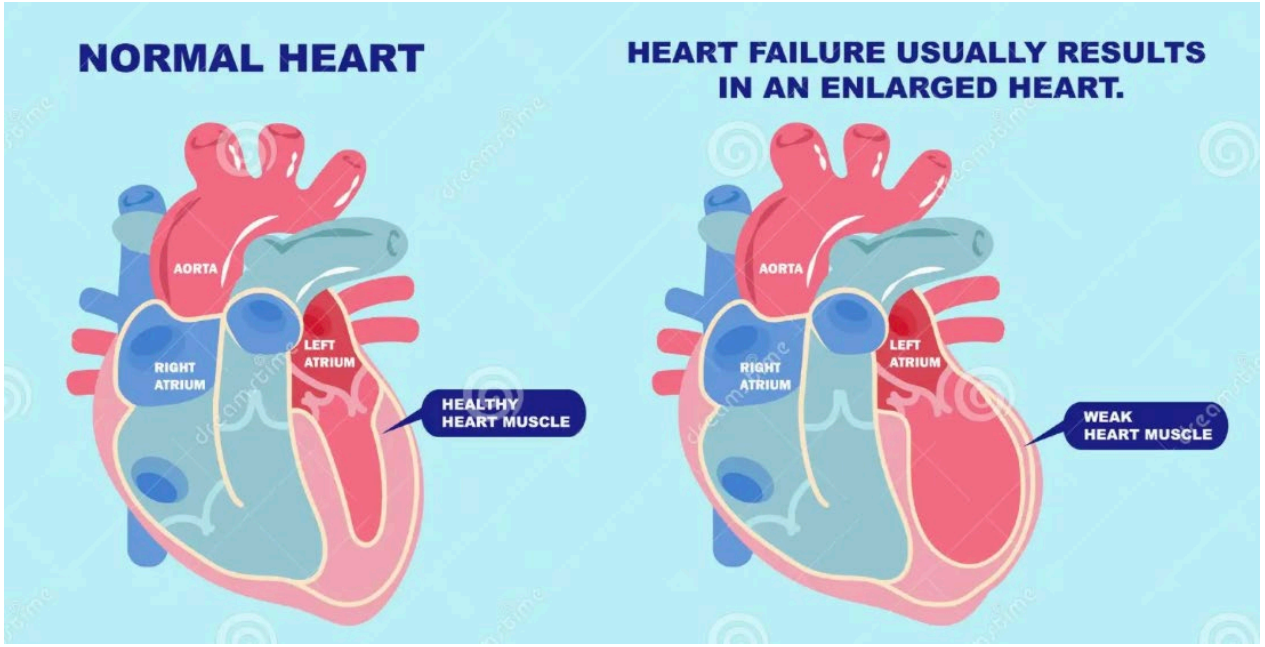
Atrial Fibrillation
(Risk is increased)

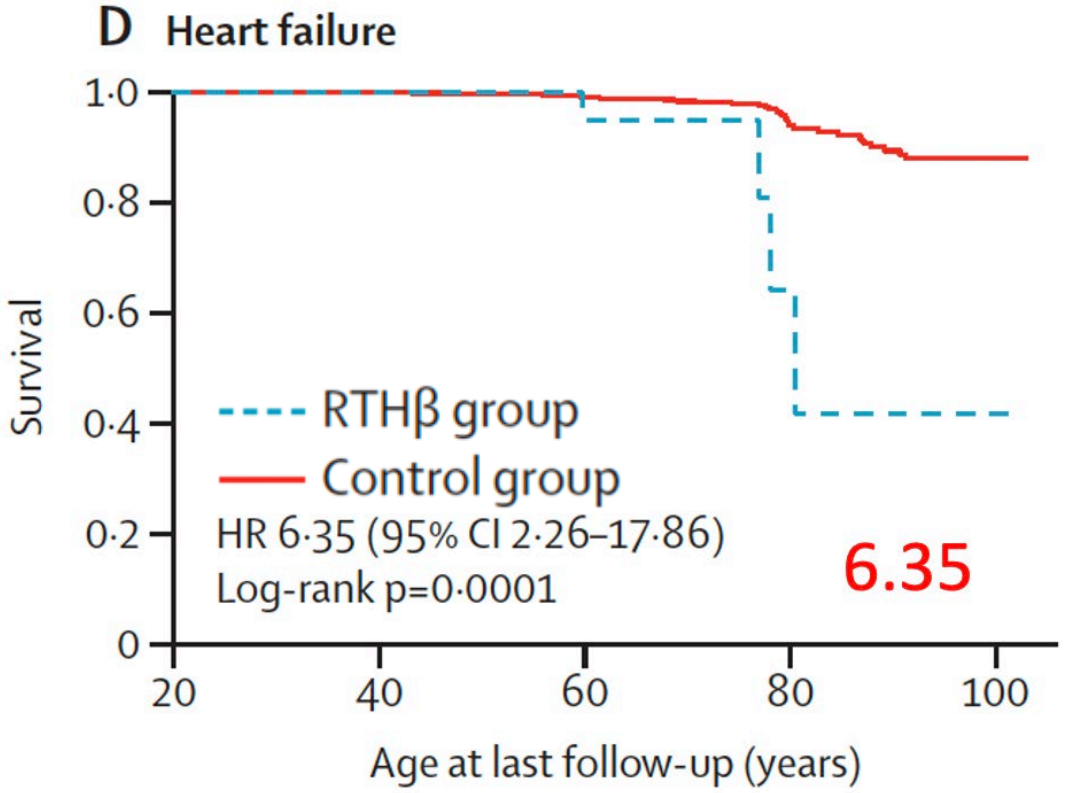




Features of RTHβ: focus on heart

Heart Failure
(Risk is increased)





Monitoring of RTHβ

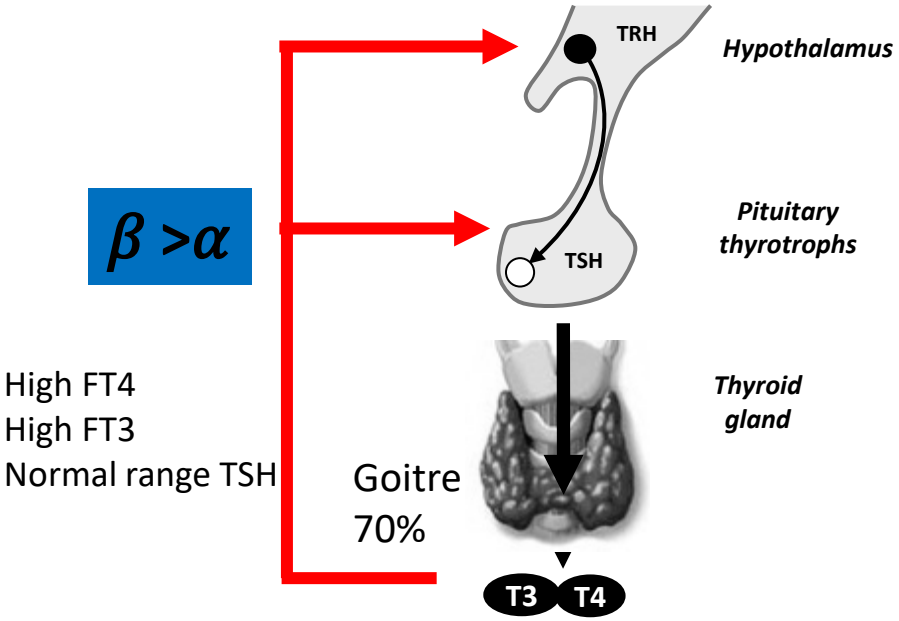
Both Children and adults
Children only
Adults only

	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

Overview

4. Treatment options, Unmet Needs

RTHβ: Treatment



Current treatment options are not optimal

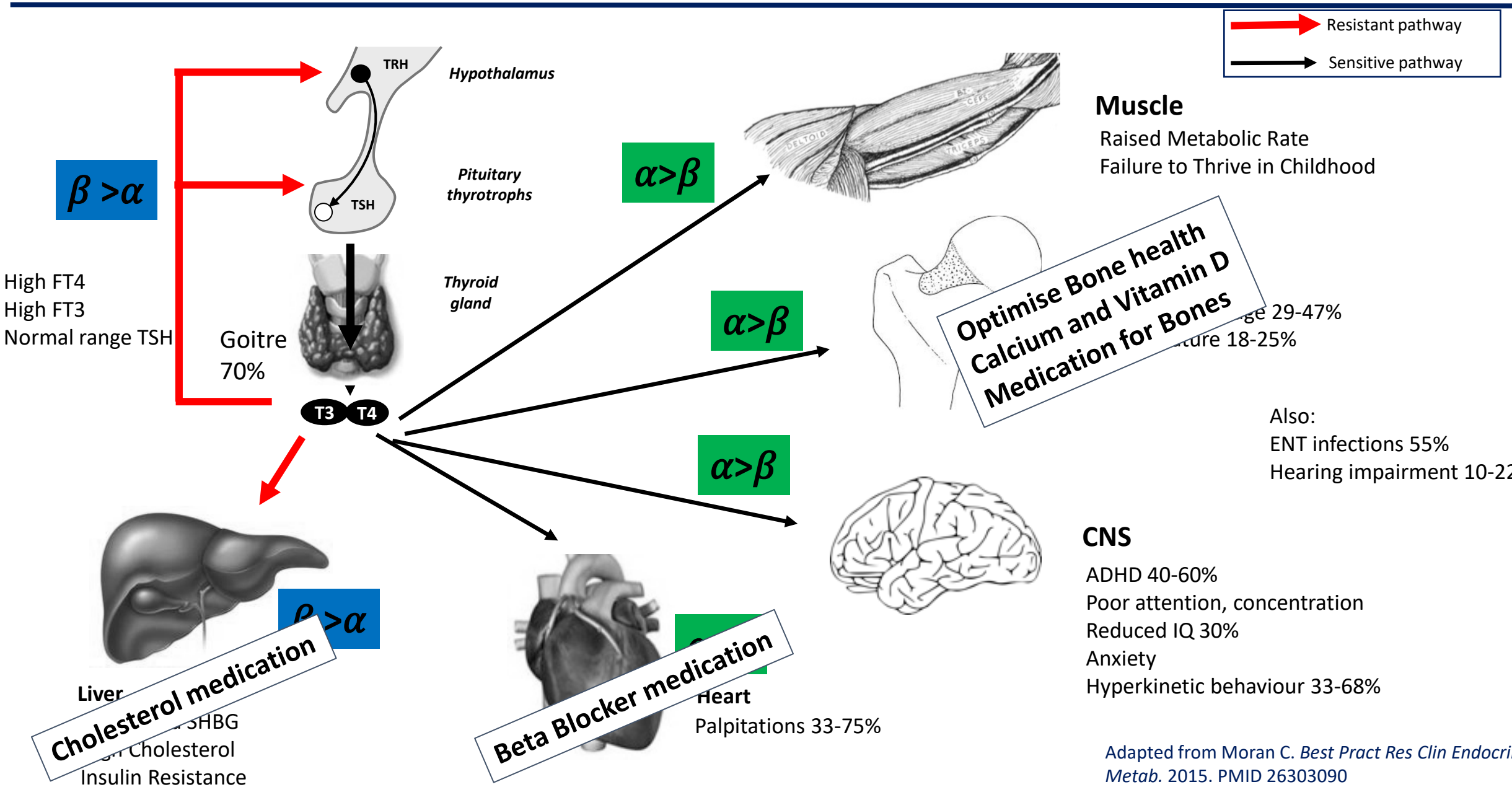
Conventional Treatments for an Overactive Thyroid Gland not recommended

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

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

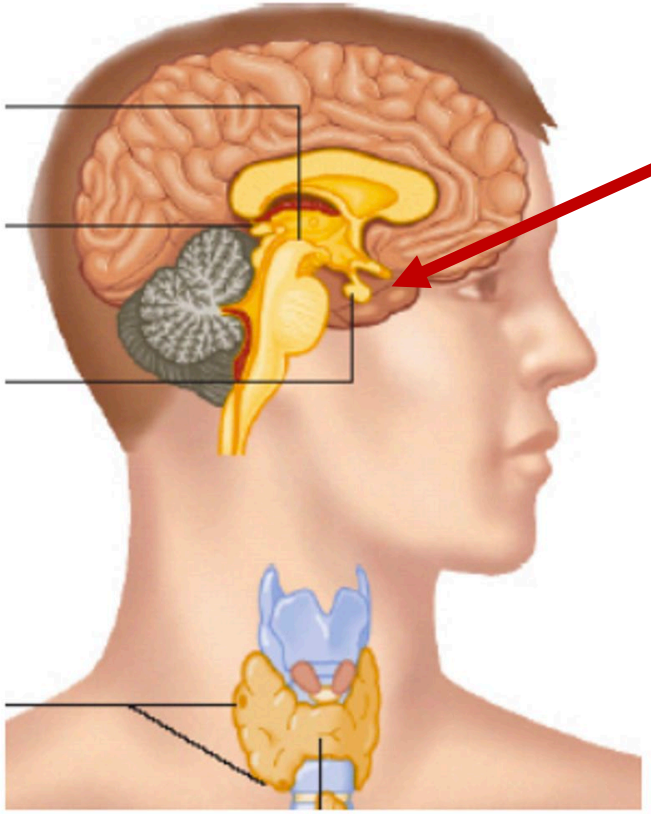
Adapted from Moran C. *Best Pract Res Clin Endocrinol Metab.* 2015. PMID 26303090

RTHβ: Treatments



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

Tiratricol



Tiratricol

Thyroid Stimulating Hormone (TSH) ↓

↓

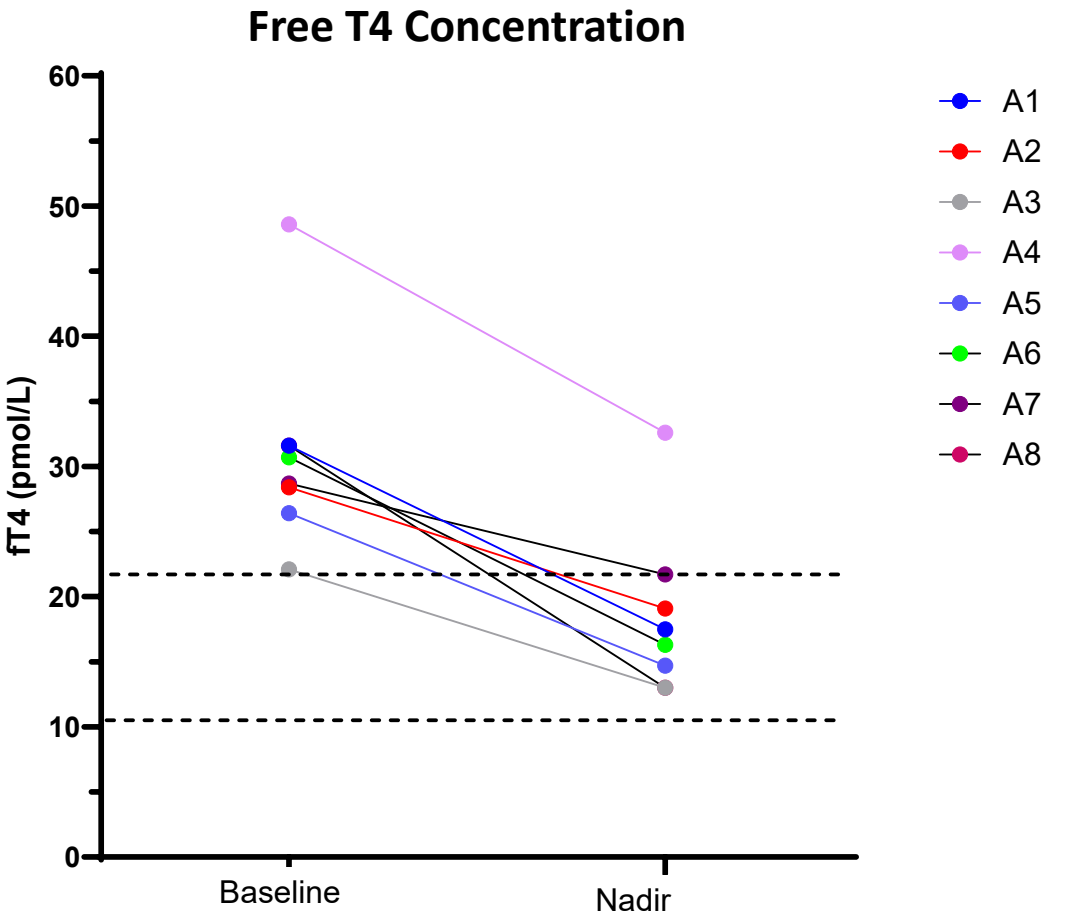
Thyroid Hormones ↓
T4 and T3

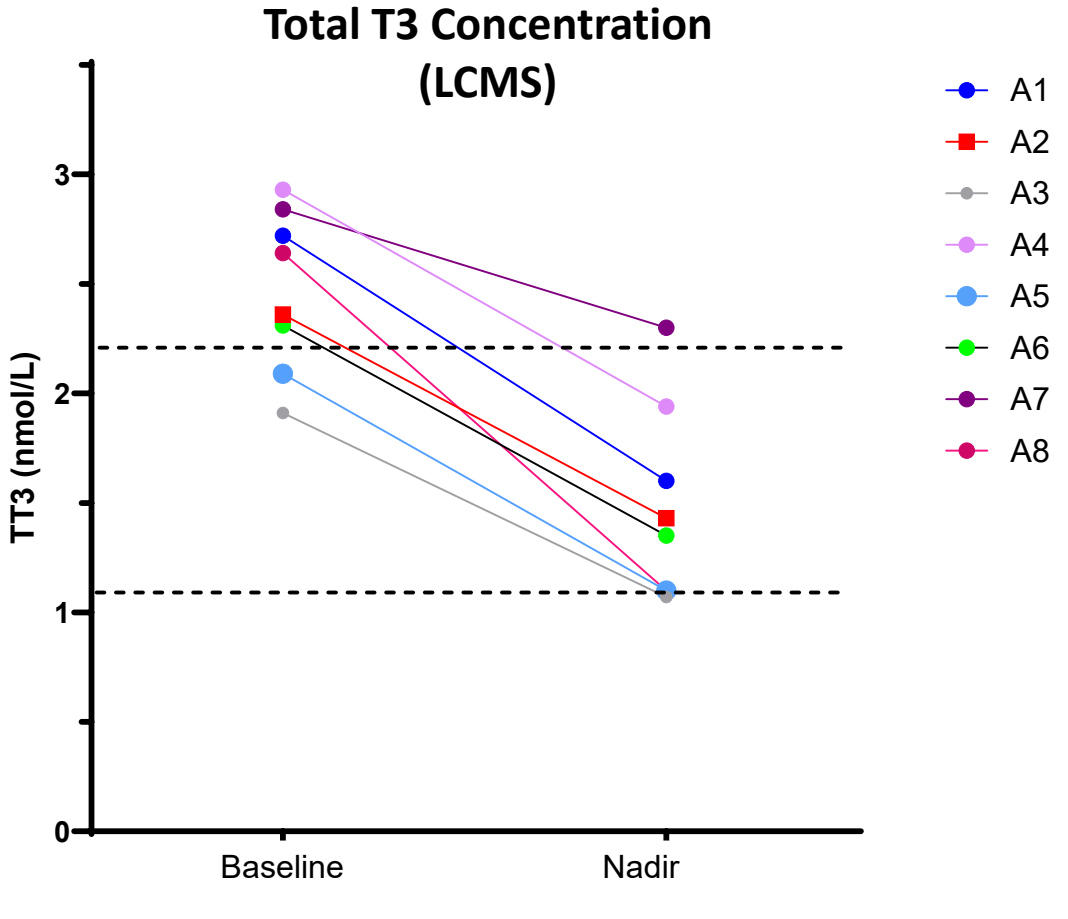
Tiratricol
= Tri-iodothyroacetic Acid
= TRIAC
= “Emcitate”

Tiratricol

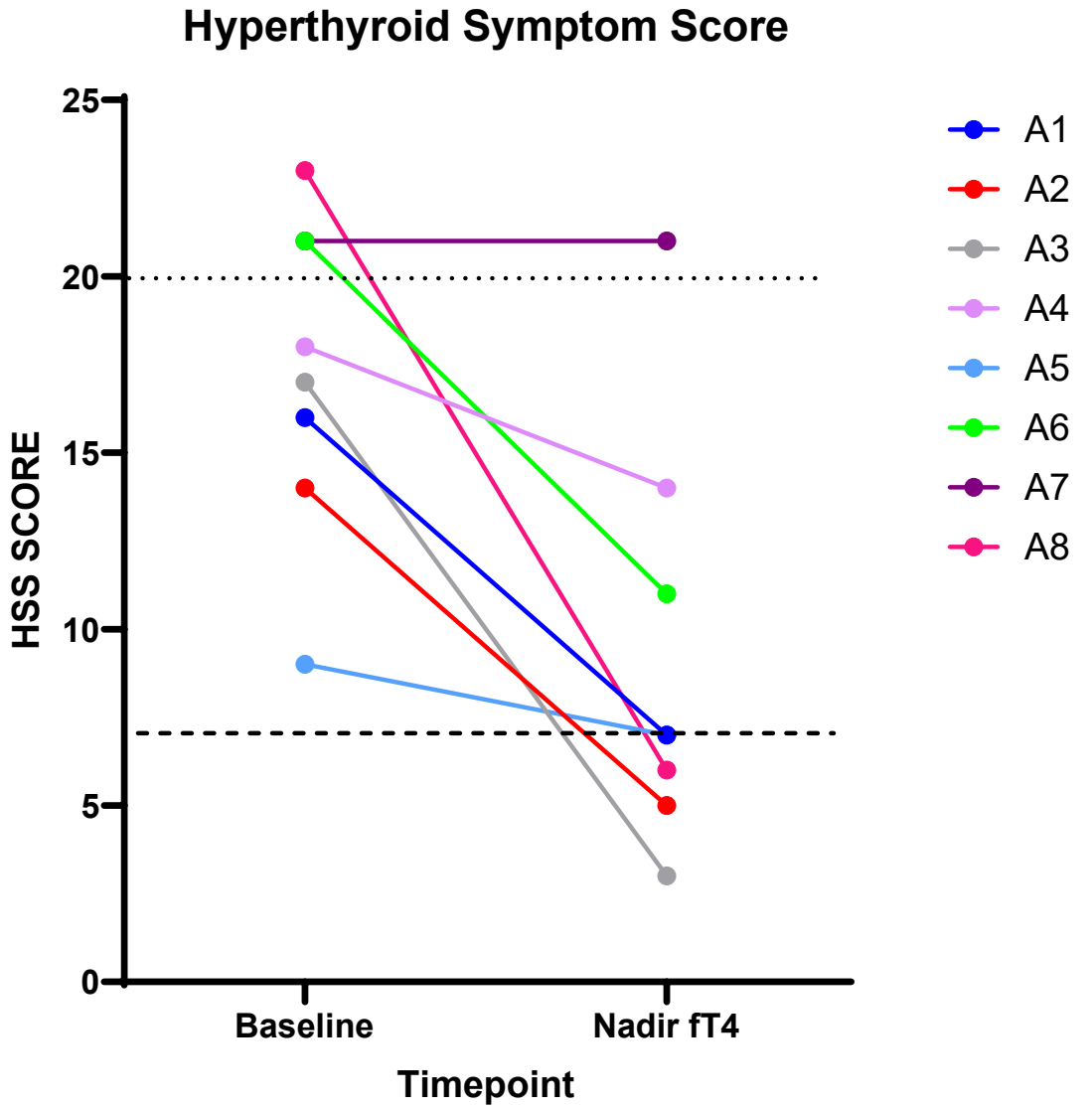
- Managed access programme at Cambridge
- 8 adults with RTHβ treated with Tiratricol monotherapy
- Mean age 36 years
- Duration of treatment 13-143 months (mean 40 months)

Thyroid Hormone Concentration on Triac Treatment



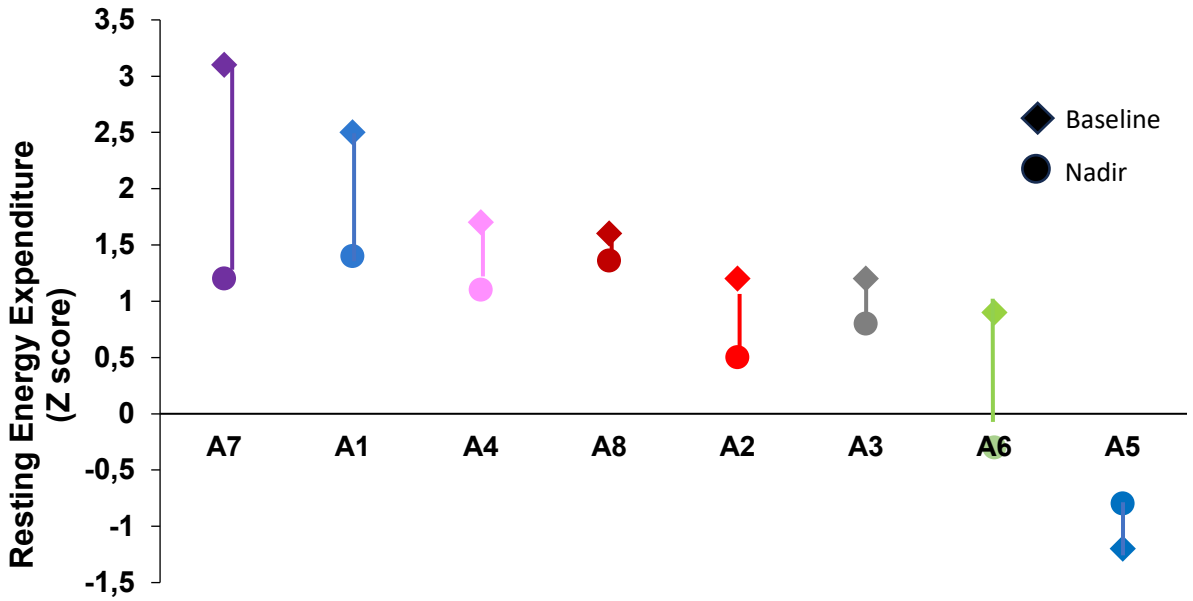


Symptoms on Tiratricol Treatment



Mean HSS Score ↓ 8 Points

Resting Energy Expenditure



Overview

6. Future Research Priorities

Patient Webinar Feedback

Health Registry

Treatments

Understanding Heart Health

CLINICAL ENDOCRINOLOGY JOURNAL FOUNDATION

Tack

Thank you

Diolch

EGETIS THERAPEUTICS

WE CARE

FOR THE RARE

Investor Day

December 18, 2024

Q&A

EGETIS THERAPEUTICS

WE CARE

FOR THE RARE

Concluding remarks

December 18, 2024

Mats Blom, Chairman of the Board

Agenda: Egetis Investor Day, December 18, 2024

Time (CET/ET)	Subject	Presenter(s)
15:00/9.00am	Welcome, CHMP opinion & corporate update	Nicklas Westerholm, CEO
15:10/9.10am	MCT8 deficiency: recent advances with tiratricol	Prof. Edward Visser, Erasmus Medical Center, NL
15:35/9.35am	Q&A	Visser & Westerholm
15:45/9.45am	Global launch preparations	Henrik Krook, Raymond Francot, Henna Oittinen-Corbinelli, Peter Verwaijen
16:20/10.20am	Q&A	Krook, Francot, Oittinen-Corbinelli, Verwaijen, Westerholm
16:30/10.30am	Break	
16:50/10.50am	US regulatory pathway & ReTRIACt study	Westerholm
17:00/11.00am	US opportunity for *Emcitate*	Anny Bedard, Ann-Marie Redmond
17:15/11.15am	Q&A	Bedard, Redmond, Westerholm
17:25/11.25am	RTH-beta and the unmet medical need	Prof. Aled Rees, Cardiff University, UK
17:50/11.50am	Q&A	Rees & Westerholm
17:55/11.55am	Concluding remarks	Mats Blom, Chairman of the Board
18:00/12.00pm	Ends	

Concluding remarks

- Egetis – a de-risked biotech with substantial unlocked potential
  - Strong data in clinical trials, demonstrating significant effects on key clinical outcomes
  - Already passed most of typical drug development risks
  - Significant market opportunity
- CHMP opinion for Emcitate® (tiratricol) for the treatment of MCT8 deficiency
  - Major step forward in building a sustainable rare disease company
- Maturing into commercial stage
  - EU launch
  - NDA submission
- Opportunity for indication expansion into RTH-beta