Clinical Trials Update

Prof Bobby McFarland

Wellcome Centre for Mitochondrial Research

Newcastle University

clinicaltrials.gov

We will be updating this site in phases. This allows us to move faster and to deliver better services.

Show less

IMPORTANT: Listing of a study on this site does not reflect endorsement by the National Institutes of Health. Talk with a trusted healthcare professional before volunteering for a study. Read more...

ClinicalTrials.gov

A service of the U.S. National Institutes of Health

Saved Studies (0)

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ClinicalTrials.gov is a registry and results database of publicly and privately supported clinical studies of human participants conducted around the world.

Search (all fields optional)

Condition / Disease: Mitochondrial disease

Other Terms: e.g., NCT number, drug name, investigator name

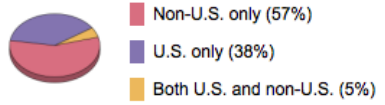
Country:

Find a study to participate in | Search all studies | Advanced Search

Help | Studies by Topic | Studies on Map | Glossary

The database currently lists 253,705 studies with locations in all 50 States and in 200 countries.

Recruiting Study Locations



44,215 recruiting studies (September 04, 2017)

More Information

For Patients and Families

For Researchers

For Study Record Managers

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clinicaltrials.gov

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Status

Studies:

- Not yet recruiting
- Recruiting
- Enrolling by invitation
- Active, not recruiting
- Suspended
- Terminated
- Completed
- Withdrawn
- Unknown status†

Expanded Access:

Eligibility Criteria

Age:

years OR

Group:

- Child (birth–17)
- Adult (18–65)
- Senior (66+)

Sex:

- All
- Female
- Male

Accepts Healthy Volunteers

Study Type

Study Results

Showing: 1-10 of **185** studies 10 studies per page

Row	Saved	Status	Study Title	Conditions	Interventions
1		Terminated	Anesthetic Effects in Mitochondrial Disease	Mitochondrial Disease	Drug: sevoflurane
2		Completed	Glycemic Index in Mitochondrial Disease	Mitochondrial Diseases	Other: test meal
3		Recruiting	Anesthesia in Patients With Mitochondrial Disease	Mitochondrial Diseases	Drug: Sevoflurane; Drug: Dexmedetomidine; Drug: Propofol
4		Recruiting	Magnetic Resonance Imaging (MRI) Muscle Phenotyping in Mitochondrial Disease	Mitochondrial Disease	
5		Completed Has Results	Phase III Trial of Coenzyme Q10 in Mitochondrial Disease	Mitochondrial Diseases	Drug: CoenzymeQ10; Drug: Placebo
6		Enrolling by invitation	Open-Label Extension Trial to Characterize the Long-term Safety and Tolerability of Elamipretide in Subjects With Genetically Confirmed Primary Mitochondrial Disease (PMD)	Primary Mitochondrial Disease	Drug: elamipretide
7		Completed	Survey on Supplement Use in Mitochondrial Disease	Mitochondrial Disease	
8		Completed	Calf Muscle Strength in Mitochondrial Diseases	Mitochondrial Disease	Other: MRI and muscle dynamometer
9		Completed	A Long-Term Extension Study of RP103-MITO-001 (NCT02023866) to Assess RP103 in Children With Inherited Mitochondrial Disease	Mitochondrial Diseases	Drug: Cysteamine Bitartrate Delayed-release Capsules
10		Recruiting	The Effect of Arginine and Citrulline Supplementation on Endothelial Dysfunction in Mitochondrial Diseases	Mitochondrial Diseases	Dietary Supplement: Arginine; Dietary Supplement: Citrulline

Showing: 1-10 of **185** studies 10 studies per page

Paging: |< < > >|

Open-Label Extension Trial to Characterize the Long-term Safety and Tolerability of Elamipretide in Subjects With Genetically Confirmed Primary Mitochondrial Disease (PMD)

This study is enrolling participants by invitation only.

Sponsor:
Stealth BioTherapeutics Inc.

Information provided by (Responsible Party):
Stealth BioTherapeutics Inc.

ClinicalTrials.gov Identifier:
NCT02976038

First received: November 18, 2016
Last updated: March 1, 2017
Last verified: March 2017
History of Changes

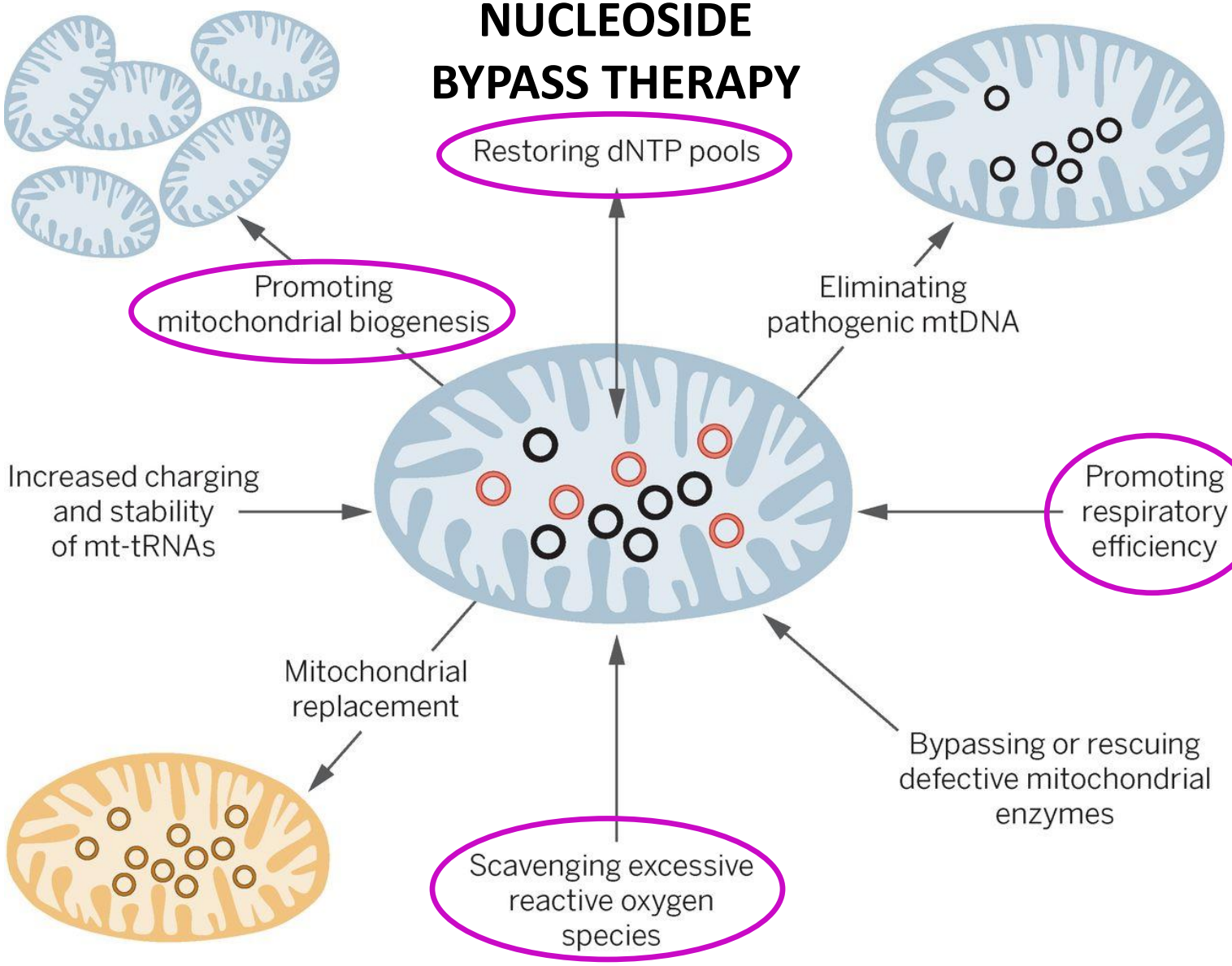
Purpose

This is a Phase 2 Open-Label extension study to evaluate the long term safety and tolerability of daily elamipretide injections in patients with genetically confirmed Primary Mitochondrial Disease who previously participated in the SPIMM-202 Clinical Trial

Condition	Intervention	Phase
Primary **Mitochondrial Disease**	Drug: elamipretide	Phase 2

Study Type: Interventional
Study Design: Intervention Model: Single Group Assignment
Masking: None (Open Label)
Primary Purpose: Treatment

BEZAFIBRATE/ OMAVELOXONE



ELAMIPRETIDE

Mutations causing mitochondrial disease: What is new and what challenges remain? Robert N. Lightowlers, Robert W. Taylor, Doug M. Turnbull*
Science 25 Sep 2015: Vol. 349, Issue 6255, pp. 1494-1499 DOI:10.1126/science.aac7516

EPI-743 for Metabolism or Mitochondrial Disorders

This study is currently recruiting participants.

See Contacts and Locations

Verified June 16, 2017 by National Institutes of Health Clinical Center (CC) (National Human Genome Research Institute (NHGRI))

Sponsor:
National Human Genome Research Institute (NHGRI)

Information provided by (Responsible Party):
National Institutes of Health Clinical Center (CC) (National Human Genome Research Institute (NHGRI))

ClinicalTrials.gov Identifier:
NCT01642056

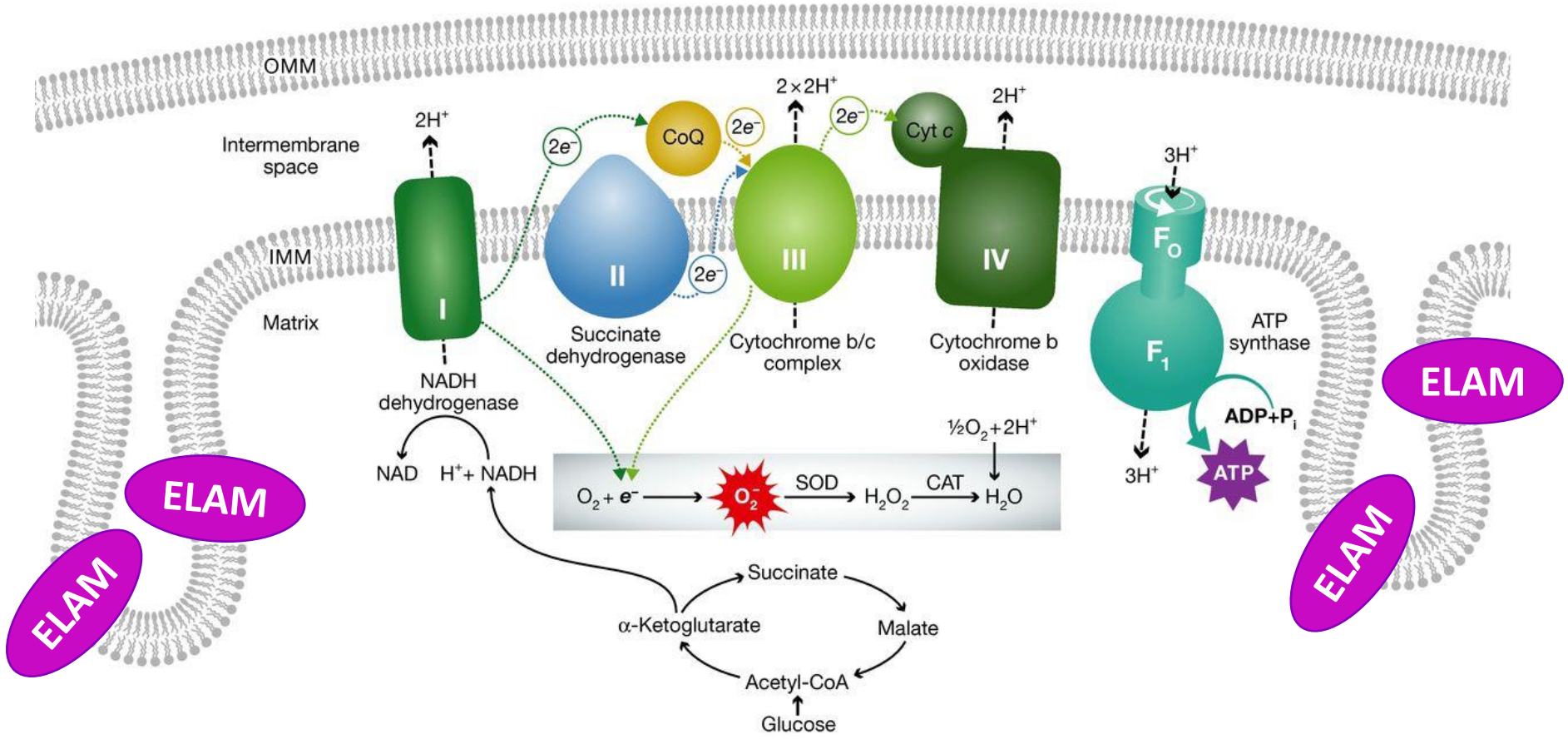
First received: July 14, 2012
Last updated: June 30, 2017
Last verified: June 16, 2017
History of Changes

Purpose

Background:

- Mitochondria are the parts of cells that help produce energy. Metabolism is the process by which the body uses energy to help cells grow and reproduce. Metabolic and mitochondrial disorders affect the body s ability to produce and store energy. These disorders can cause a wide variety of problems, but most often they affect the muscles and the brain, where energy requirements are high. Treatment is difficult because the exact source of the problem is hard to detect.
- EPI-743 is a new drug that is based on vitamin E. Tests have shown that it can help improve the function of cells with mitochondrial problems. It may be able to treat people with genetic

Elamipretide – Interaction with Cardiolipin



Elamipretide (aka Bendavia or Ocuvia)

- SPIMM-203: '**MMPOWER-2**'
  - Open label extension of Phase 2 study in patients with PMD and myopathy.
  - Currently has ~30 patients continuing on treatment with elamipretide.

- SPIMM-300: '**RePOWER**' Phase 3 database.
  - Currently enrolling patients with confirmed PMD and predominant myopathy
  - International database - 34 targets sites across the world.
  - Must be enrolled in this database to be randomized into the Phase 3 clinical study.
  - Ages ≥ 16 yr and must be able to perform the 6MWT. Target of 300 subjects.

- SPIMM-301: Phase 3 clinical study expected to start in Q4 2017.
  - A randomized, double-blind, placebo-controlled study.
  - Will enroll approximately 200 patients with primary mitochondrial myopathy.

- Other studies in Barth, LHON and heart failure

Stealth BIOTHERAPEUTICS | Leading Mitochondrial Medicine

Omaveloxolone aka RTA 408

- Mitochondrial biogenesis (+ antioxidant + impro
- Nrf2 activator: Nrf2 regulates genes involved in
- Phase II study – MOTOR - in patients with mitoc
- EXERCISE testing
- 12 weeks (2+10) dose escalation - randomised double blind placebo
- Adult patients (18-75yr)
- Actively recruiting – aiming for 100 participants. Closing Dec 2017?
- Outcome measures
  - Primary - Change in peak workload in exercise testing
  - Secondary – Change in distance walked in 6MWT
- USA and Denmark

REATA PHARMACEUTICALS

Bezafibrate aka Bezalip

- Repurposed drug: designed to lower fat (Triglycerides and LDL)
- Activates PGC1α and increases mitochondrial biogenesis
- Can cause a muscle inflammation in people with kidney problems
- Phase II study complete
  - Intended to recruit 10 patients with mitochondrial myopathy and m.3243A>G
  - Open label study – proof of concept, safety and tolerability
  - Muscle biopsy at the beginning and end of study (12 weeks)
  - Participants took 200mg (x3/day) for 6 weeks then 400mg (x3/day) for 6 weeks
  - 6 patients completed the study
  - Results in analysis but well tolerated and appears to be safe
  - Aim to undertake a larger DBRCT

MERCK
INVENTING FOR LIFE

RP103 aka Cysteamine Bitartrate aka Procysbi

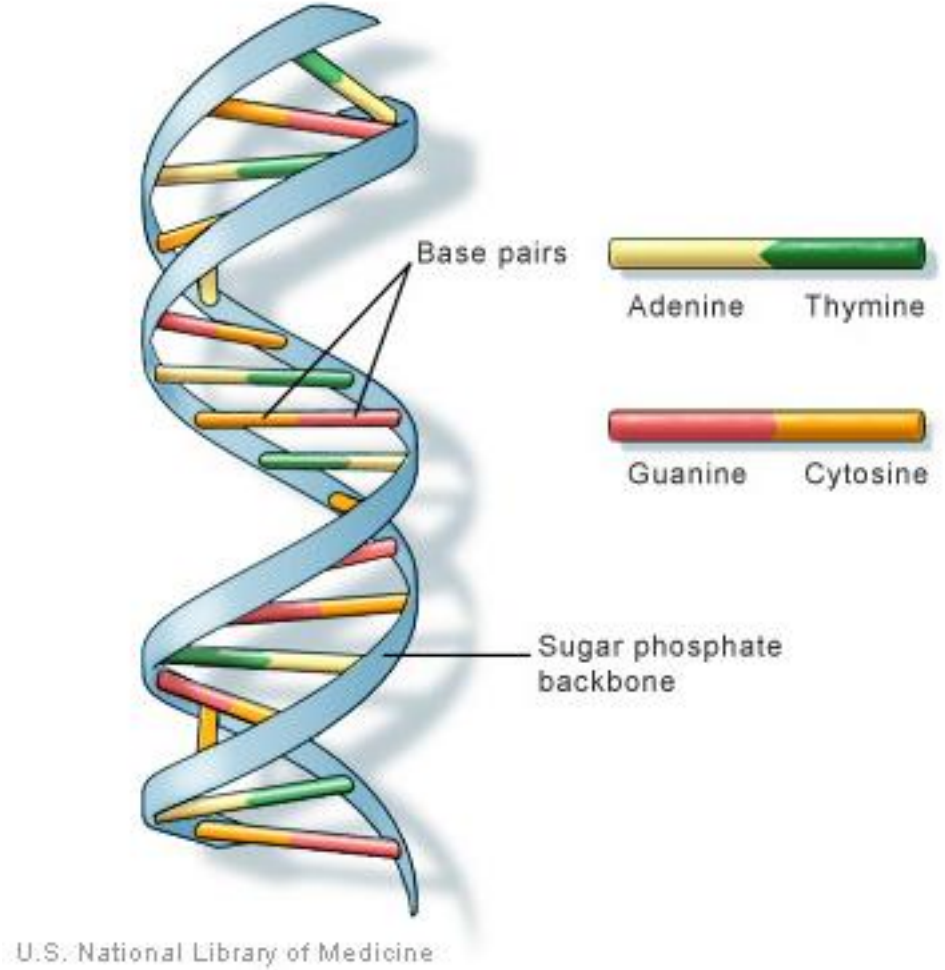
- Cysteamine Bitartrate Delayed-release Capsules (RP103)
- Prescribed for nephropathic cystinosis
- Side effects on skin, bone, bowel and brain noted in these patients
- Raptor Pharmaceuticals trialled RP103 in 2014 with extension in 2015
- Phase 2, open label to assess safety, tolerability and efficacy
- No longer recruiting. No results available.
- Horizon Pharma acquired Raptor for $800 million in autumn 2016

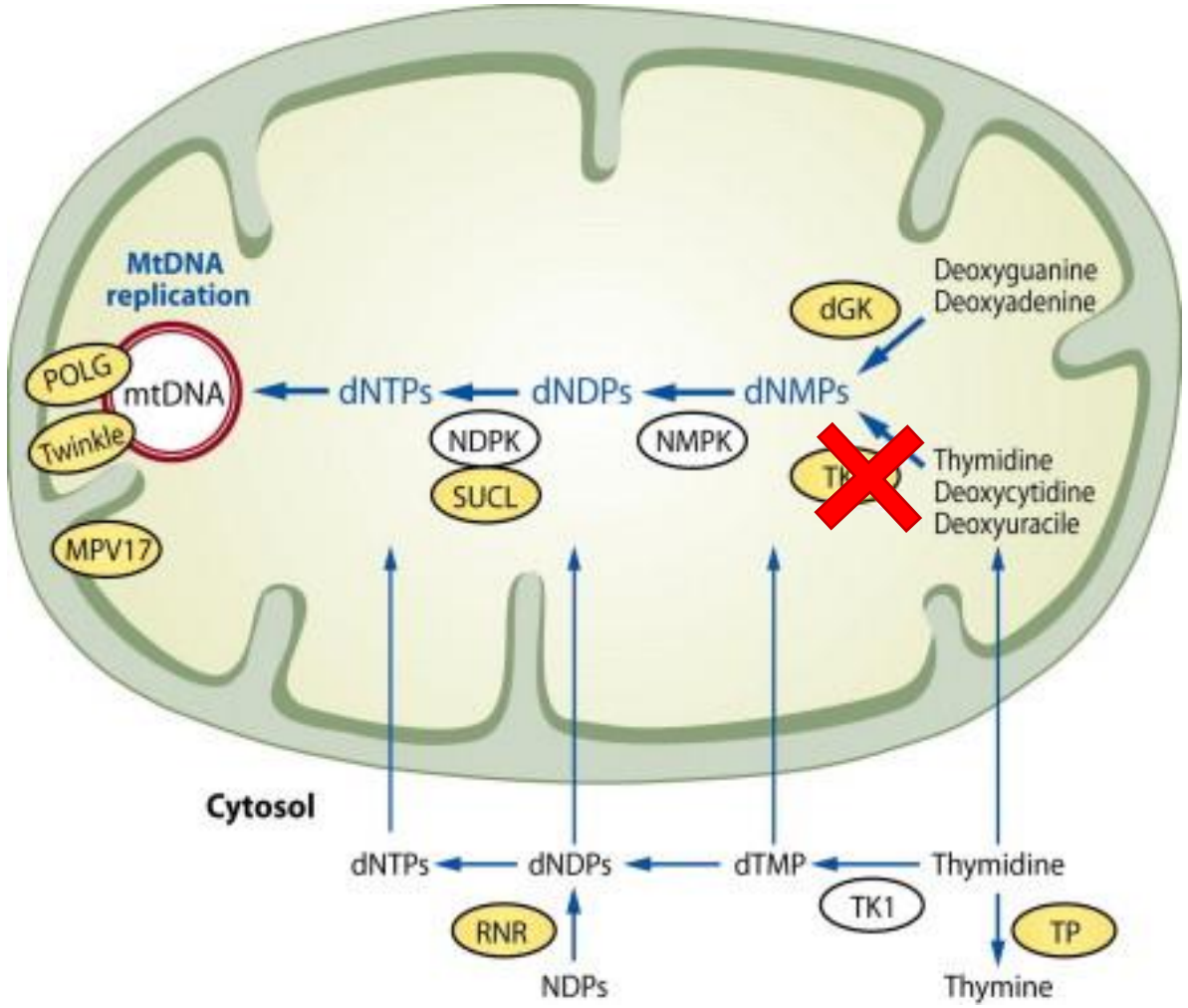
KH176

- New class of drug – helps controls redox status
- Phase I (healthy volunteers) study completed in Dec 2015
- Phase II (patients) KHENERGY study
  - Adults with m.3243A>G and clinical disease
  - 20 patients
  - Double blind randomised placebo controlled study
  - Results will become public by the end of the year/early 2018
- Phase II KHENERGYC
  - Children with mitochondrial disease

KHONDRION

Nucleoside bypass therapy





Mitochondrial DNA depletion syndromes--many genes, common mechanisms. Suomalainen A, Isohanni P. Neuromuscul Disord. 2010 Jul;20(7):429-37. doi: 10.1016/j.nmd.2010.03.017. Epub 2010 May 4. Review. PMID: 20444604

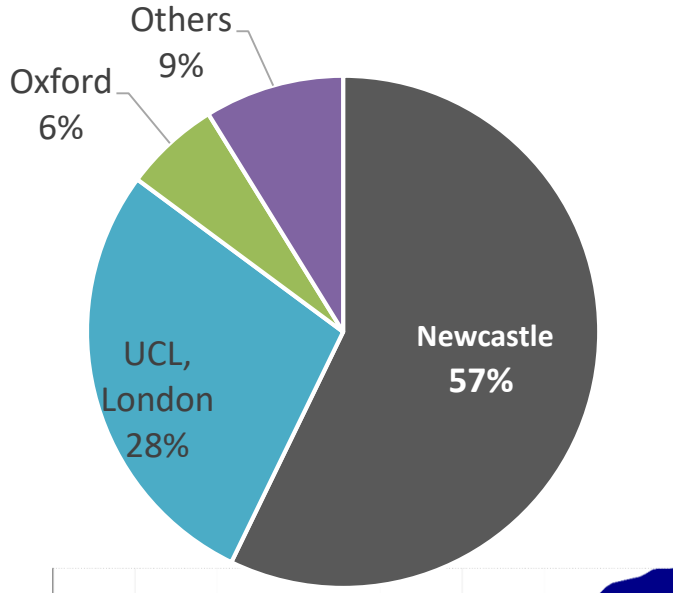
TK2 therapy in patients

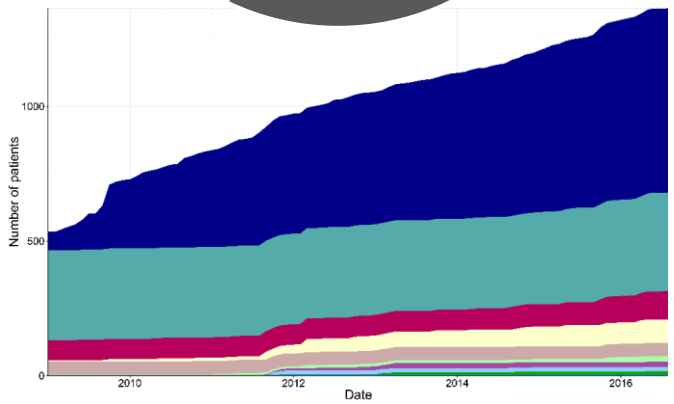
- 18 patients with TK2 deficiency have been treated with dTMP+dCMP (1), dT+dC (11), or both sequentially (6). Total cumulative exposure 45 years.
- 4 infantile-onset; 10 childhood-onset; 4 late-onset
- Ages at initiation of therapy: 1-58 years-old
- Current ages: 3-60 years-old
- Publication of results soon

Dr Caterina Garone on behalf of the International TK2-deficiency Treatment Consortium

MitoCohort Recruitment

Patient Recruitment (n=1471; NCL 882)





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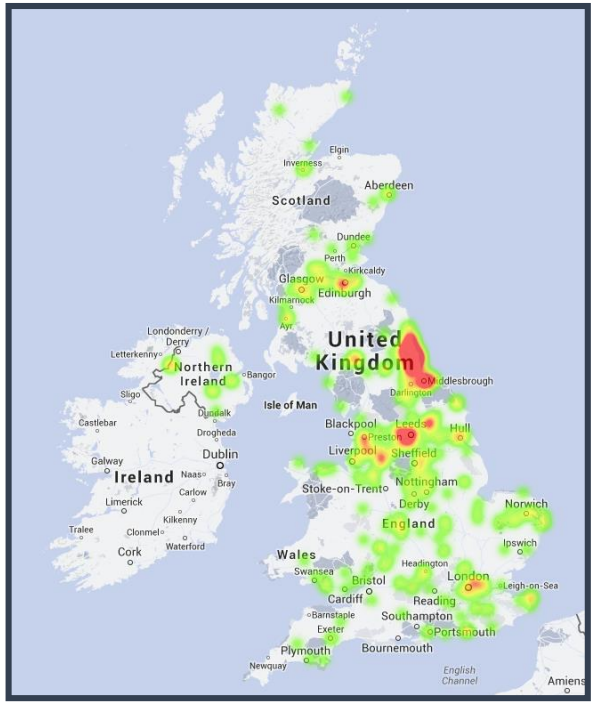

Alex Bright