MITO CAMB

MitoCAMB Patient Newsletter

Issue 10
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UNIVERSITY OF CAMBRIDGE

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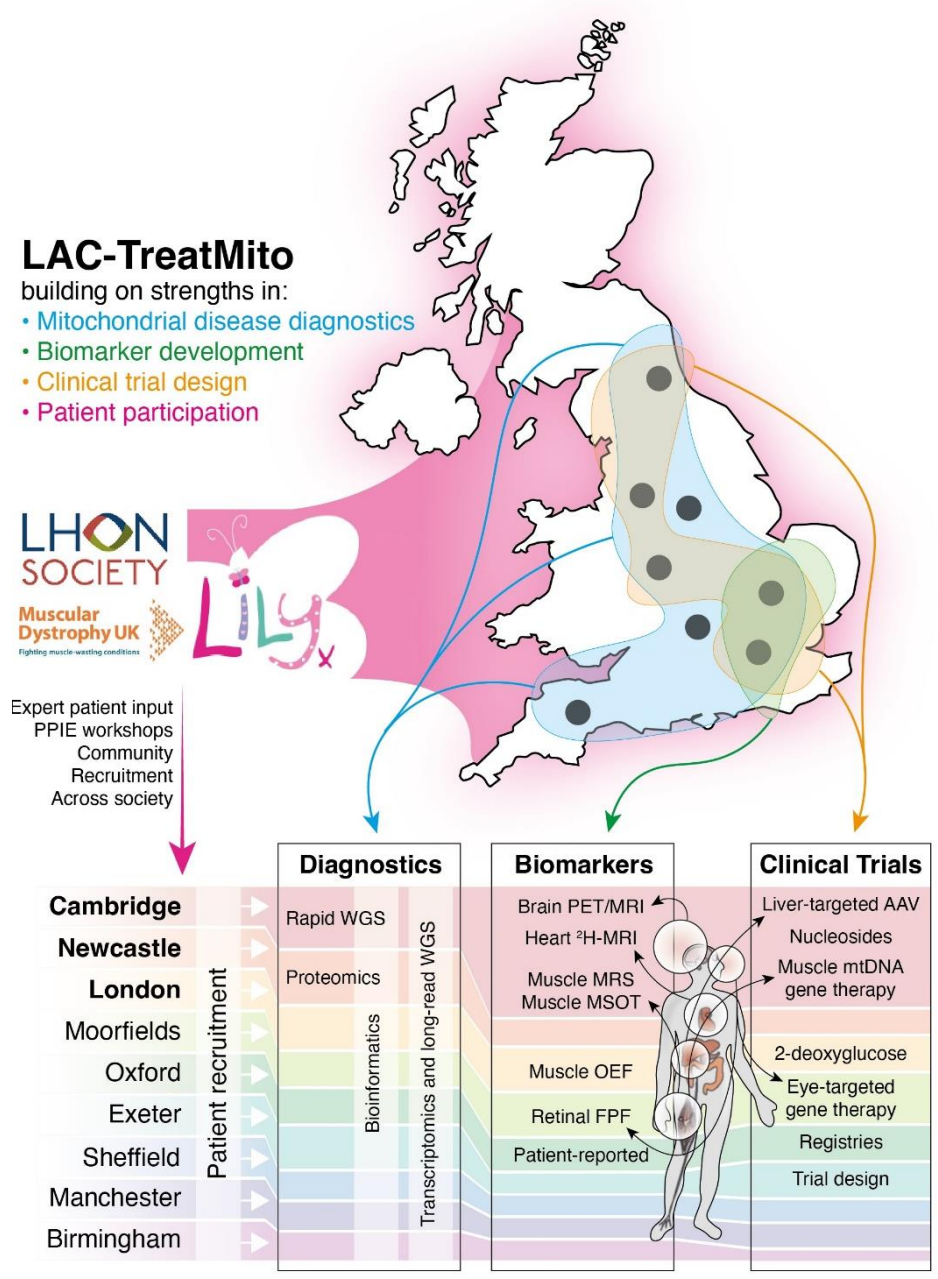
WELCOME

…to our Cambridge Clinical Mitochondrial Research Group patient newsletter.

You are receiving this because you have previously signed up to help with research into mitochondrial disease or neurodegenerative disorders. Our aim is to provide you with the latest news from the research group, giving you more information on who we are, what we do, the science behind our work, and how we can support you, our patients.

LIFE ARC CENTRE TO TREAT MITOCHONDRIAL DISEASES (LAC-TreatMito UK)

In April, LifeArc announced that they would be launching 4 Translational Centres for Rare Diseases, spending £40 million over 5 years, and we are delighted to be leading one of them: the LifeArc Centre to Treat Mitochondrial Diseases (LAC-TreatMito UK).



Funded by LifeArc and Muscular Dystrophy UK (MDUK), LAC-TreatMito-UK is the first national mitochondrial disease treatment platform worldwide, aiming to connect specialists, patients, knowledge and infrastructure. It is a collaboration between the University of Cambridge, University College London, Newcastle University, and other centres (including Oxford, Birmingham and Manchester), in full partnership with The Lily Foundation.

Our over-arching aim is to develop new treatments for rare mitochondrial diseases.

The three main aims of the centre are:

Diagnostics – to increase the proportion of patients and families receiving a genetic diagnosis for mitochondrial disease.
Biomarkers - to build a portfolio of clinically-relevant biomarkers specific to organ types to evaluate new treatments.
New treatments – to show that the national platform is effective through the delivery of new therapies.

None of this will be possible without patient participation, and our aim is to work together with patients and their families to act as equal partners to help shape the centre.

We will share more information on this exciting development in future issues, but if you would like to read the press release, it can be found here: https://www.lifearc.org/2024/lifearc-launches-40m-research-centres-that-will-unlock-new-tests-treatments-and-cures-for-people-living-with-rare-diseases/

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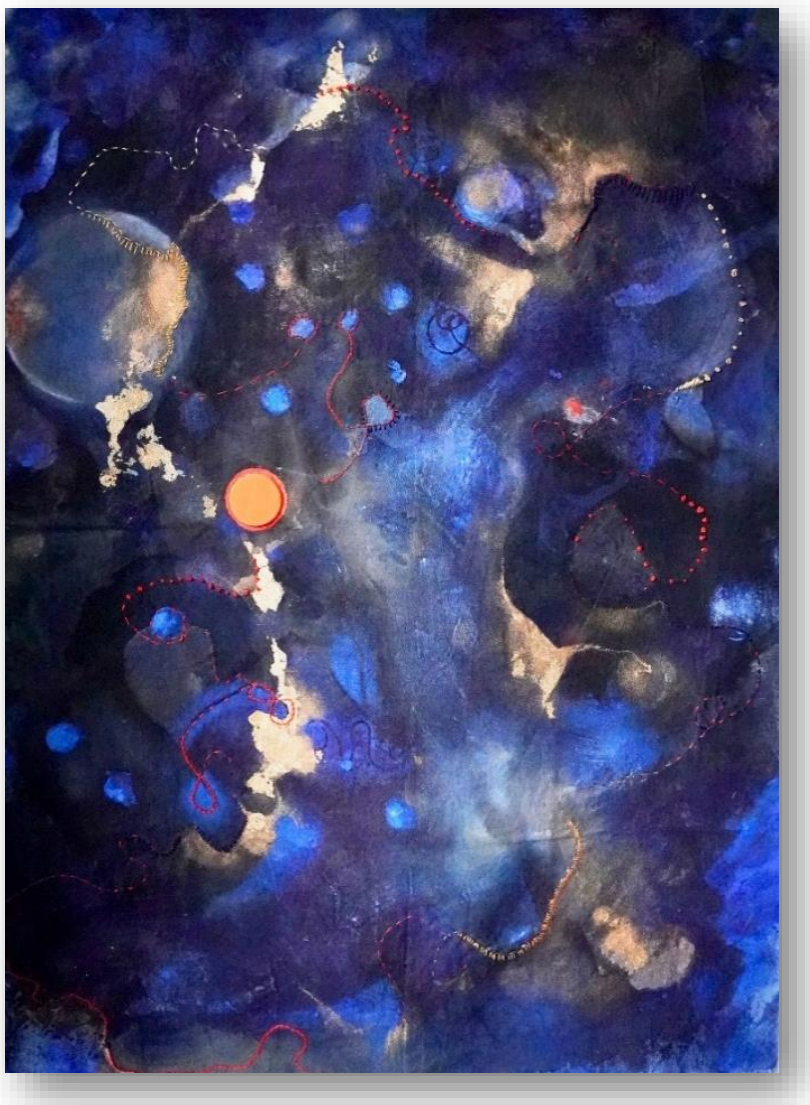
ART TRANSLATIONS



The ART-TRAnslations project is helping to bring research and science to life through art. It was launched at the LifeArc conference in April and will be transferring to Rarefest later in the year.

The MitoCAMB team was paired with Kelly Briggs, an emerging artist, who produced the artwork featured here, inspired by mitochondrial disease. The artwork is an interactive tactile map of how difficult mitochondrial disease is to diagnose.

The piece was displayed at the Life Arc conference in London as a part of a wider exhibition of other artists responding to rare diseases. It was sold to a collector in Geneva, with half the proceeds of the sale going to the Lily foundation!

As the artwork has already been sold, Kelly is busy producing a new piece specifically for Rarefest!

To see more of Kelly's work go to: www.kelly-briggs.com
instagram artist_kellybriggs

camRARE
Cambridge Rare Disease Network

DefiNe TRIAL

A trial to test the use of deferiprone in people with neuroferritinopathy. This trial is now officially open to recruitment! Thank you to everyone for your patience.

We will be contacting patients in the coming weeks to invite them to take part, but please do spread the word.

As a reminder, the key facts are:

- Deferiprone is used to remove excess iron from the body. It has been shown in a related disorder that deferiprone is able to reduce brain iron and slow clinical progression, and we now want to test this in people with neuroferritinopathy.
- The DefINe trial is comparing two groups: 1) a group who receive deferiprone, and 2) a group who will receive a placebo, or 'dummy' drug. Participants will be randomly allocated to one of the two groups and they will have a 50% chance of receiving deferiprone.

- Overall participation in the trial will last for 13 months, including 4 in-person visits at Addenbrooke's Hospital in Cambridge, and 3 MRI scans.
- In between in-person visits, participants will be contacted on a monthly basis by the trial team and will undergo regular blood testing for safety reasons.
- Travel expenses will be reimbursed.

LifeArc

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LILY FAMILY WEEKEND 2024

The Lily Family weekend took place over a sunny weekend in June and various members of the MitoCAMB team joined the fun! On Saturday, Dr Jelle van den Ameele, Elizabeth Ashby and Heather Biggs provided information about the new LAC-TreatMito UK centre and the key aims for research. They also engaged with parents and patients to help direct the science and research, and assess how and what to communicate about the centre with patients and their families.

On Sunday, scientists from the MRC Mitochondrial Biology Unit (MRC-MBU) Dr Michele Frison, Eleni Theiaspra and Rajini Chandrasegaram (pictured) entertained and educated with 'electron transport chain pinball', mitochondria shaped containers with red and green balls to sort through the 'bad' mtDNA, and play-doh for families to make their own mitochondria to take home (along with lots of MRC-MBU goodies)! The research team said that it was a wonderful experience and they would encourage anyone who works on mito disease to take part!

CONTACT US

Thank you for your continuing participation in our research programme. If you have any queries relating to research studies that you have taken part in, or if you would like further information on any of our studies, please contact the team on:

add-tr.mitoteam@nhs.net 01223 335106 @cam_mito

https://www-neurosciences.medschl.cam.ac.uk/mitocamb/

For queries regarding routine NHS clinic appointments, please contact Katrina Dedman: cuh.mitocambclinic@nhs.net or 01223 216751

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