CGDSociety

Supporting families affected by chronic granulomatous disorder

CGD Society May news

Dear CGD Society member,

Welcome to our latest news updates. We also send a warm welcome to new members of our charity and hope that you have all been keeping well.

Latest news

Gene Therapy update

The following statement is provided by our chair of trustees, Geoff Creamer:

"This month we were informed by Orchard Therapeutics that their work to develop Gene Therapy for Primary immunodeficiencies including CGD will be ended. This is driven by their need to reduce costs and improve their results in the light of investor reluctance to provide more funding. Their headcount will reduce by 30%.

We already knew that the early stage 1 CGD trials showed reduced effectiveness over time for some patients, so the stage 2 trials had been postponed but this news is nonetheless a disappointment after nearly 4 years of collaboration. It reminds us that whilst Gene Therapy remains an essential target for CGD care, the development path is difficult and the outcomes uncertain. The current protocol has been 30 years in the making, with significant investment from this charity, but we are still several years away from a clinic-ready treatment.

Orchard is looking for partners to continue this development and we are aware of continuing small-scale trials in the US which we will follow up. We hope for more positive news for our members in the near future as we continue to work towards a cure for all with CGD."

As stated by Orchard Therapeutics in a press release:

"To support the company's refined strategic focus and provide runway extension into 2024, Orchard intends to discontinue its investment in and seek alternatives for its programs in rare primary immune deficiencies. These include OTL-103 for the treatment of Wiskott-Aldrich syndrome (WAS), OTL-102 for X-linked chronic granulomatous disease (X-CGD) and Strimvelis®, a gammaretroviral vector-based gene therapy approved in Europe for adenosine deaminase severe combined immunodeficiency (ADA-SCID)"

"We recognize the significant need that persists for many patients suffering from these rare diseases of the immune system, and we sympathize with the individuals, families and healthcare providers affected by these announcements, as well as our clinical partners and colleagues who worked so hard to advance these programs. These therapies have shown the potential for significant benefit for many patients treated in the clinical studies and we will continue to look for alternative ways to advance them, which could include commercial partnerships"

The full press release can be found here.

Transition to adult care

On the 15th of October 2021, Costello Medical, in collaboration with Cambridge Rare Disease Network (CRDN) and Beacon, hosted a roundtable meeting to discuss the current challenges associated with the transition of young adults with rare diseases into adult healthcare services, as well as identify potential solutions and recommendations for action.

Read on for the full report: Transitions - Cambridge Rare Disease Network (camraredisease.org)

Charity restructure

"I am delighted to tell you all that Claire Jeffries, our Fundraising and Operations Manager has moved into a new role as Executive Director as part of our reorganisation plans for 2022. Claire has been with us for four years and has been responsible for re-energising our engagement with members through social media, newsletters and our helpline service. I hope you agree that our contact with you has improved significantly and we know that Claire has been responsible for that change. Thank you Claire.

There is always more to do so Claire will now take a more strategic role steering the activities of her team to achieve the objectives agreed with the Trustee Board. We have exciting plans for the year and Claire will be central to their delivery. Look out for more personnel announcements through the year as we recruit to fill the gaps left by Adelle and Susan who both left us last year."

Geoff Creamer, Chair.

Thank you to fundraisers

We really do have the best supporters. Without them we simply would not be able to offer the information and support we provide so many of the CGD community. We would like to give thanks to...

Emma Wormull who has taken on the challenge of walking, running and jogging 100 miles in May. Emma's son Mason underwent a BMT (Bone Marrow Transplant) 5 years ago for CGD and Emma wanted to mark the anniversary by raising much needed funds to our charity.

You are doing a wonderful job Emma! Please visit Emma's JustGiving page.

Nev Osman is taking part in this year's 25K Peak district Challenge on the 9th – 10th July in memory of her sister Reyhan who sadly passed away from complications from CGD. Nev had a stem cell transplant in 2007.

Good luck Nev. Please visit Nev's JustGiving page.

Chris Hutchings is taking on the mammoth task of cycling 980 miles from Land's End to John O'Groats from the 10th of September – 18th September. Chris's cousin sadly passed away with CGD last year.

Chris is doing well with his training and has already raised £450 of his £2,500 target. Keep up the good work Chris. Please visit Chris's JustGiving page.

If you would like to get involved in fundraising like our fabulous fundraisers mentioned, then please do get in contact at Hello@cgdsociety.org. If fundraising is not your thing, then maybe you would consider making a small donation here. Your support is deeply appreciated. You will be making such a positive difference to those affected with CGD. Thank you.

We hope you enjoyed reading this edition of our latest news.

Best wishes,

Claire, Helen and Trustees

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