Executive Director Update: Funding Promising Research Grants

Dear Cure JM Families and Friends,

At Cure JM, we have always believed that research for a rare disease like juvenile myositis can be at the cutting edge of science. Why shouldn't our JM children have not only the best of care, but also the foremost attention of the world's leading children's research hospitals working diligently to find better treatments and a cure? The rarity of the disease belies our impact, and for that, I can thank Cure JM families everywhere as the reason for our progress.

Global Recognition For Research Innovation

In fact, the strength and resolve of our families, including yours, is a major reason Cure JM and several partner institutions have just been awarded a $2 million grant from the prestigious Chan Zuckerberg Initiative. Through this grant, Cure JM researchers will investigate what changes occur at the cellular level as children with JM progress through stages of various treatments. As we better understand what happens in individual muscle and skin cells, we can better target therapies to those affected cells. This funding will go to collaborative partners such as the University of Michigan and Cure JM Centers of Excellence at UC San Francisco and Duke University.

Congratulations to Dr. Jessica Turnier at Michigan, Dr. Jessica Neely at UCSF, as well as to Cure JM Chief Scientific Officer Dr. Andrew Heaton, who serve as co-principal investigators for the grant. The Chan Zuckerberg Initiative awarded five grants in this rare disease category from dozens of competitive applications spanning the globe.

Abatacept Trial Concludes With Favorable Results

An important clinical trial to evaluate the drug abatacept for effectiveness in treating children and adolescents with JM has just concluded at the Cure JM Center of Excellence at George Washington University. The drug, which goes by the trade name Orencia, was shown to be both safe and effective in treating JM, as all but one patient with active dermatomyositis saw measurable improvement in both skin condition and muscle strength after 24 weeks on the drug. Abatacept was generally well tolerated with minimal side effects, and the preliminary data suggest that abatacept may be beneficial in the treatment of hard-to-treat JM patients. **Importantly**, patients using abatacept improved at the same time prednisone dosage was reduced.

Heartfelt thanks to Center Director Dr. Rodolfo Curiel, Dr. Gulnara Mamyrova, and Dr. Lisa Rider, chair of Cure JM's Medical Advisory Board, for this stunning success.

I don't mean to be dropping so many names here, but it is hard not to when your organization has such a stellar cast of doctors and researchers whose work we have supported over the years.

2023 Research Trials and Grant Funding

We hope clinical trials for two other drugs, baricitinib and vamorolone, both with the potential to treat JM, will be underway in 2023. Vamorolone is a new alternative steroid—one with far fewer side effects than prednisone.

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As exciting as these clinical trials for better treatments are, to actually cure juvenile myosotis, we must better understand what causes JM in order to develop interventions to cure it. Our most recent round of 2022 grant funding resulted in four substantial awards to Ohio State University/Nationwide Children's Hospital, Baylor College of Medicine, Duke University, and the Harwell Science and Innovation Campus in Oxfordshire, UK.

I'll discuss these four awards in a future Executive Director's Report, but do want to note our newest international partnership with the Harwell Campus in the United Kingdom. Harwell is the UK's most significant research institution and can best be described in the U.S. as a combination of NIH and the Manhattan Project. Medical research at Harwell is overseen by the UK's Medical Research Council and resulted in innumerable breakthroughs, including the discovery of the structure of DNA and the development of penicillin. The research of 32 Nobel Prize winners has been sponsored by the Medical Research Council. Perhaps needless to say, we're honored with this new association with Harwell.

Cure JM is funding a juvenile myositis project at the Nucleic Acid Therapy Accelerator, a new hub at Harwell to discover "nucleic acid" therapies—therapies at the genetic level--for multiple diseases. It's a different approach to gene therapy. Nucleic acid therapeutics target the genetic cause of disease to achieve long-term remission or even a cure.

Some 11 rare-disease drugs based on nucleic acid therapies have been approved by the FDA and its counterparts in Europe and Japan, and so this technology to change gene expression or function is already proven. I'm optimistic that we will see great progress in JDM.

Congratulations to Dr. Joanna Parkes at the Harwell Campus for this groundbreaking work. Dr. Parkes's work in myositis began through a fellowship at the State University of New York at Binghamton under the direction of Dr. Kanneboyina Nagaraju, a member of Cure JM's Medical Advisory Board.

I'd like to thank you and all of our friends and families for making Cure JM's work possible. We simply could not fund this and future high-level research leading to a cure without your support. If you have already contributed to this year's Giving Tuesday Holiday Challenge, **THANK YOU!** And if you have yet to contribute, there is still time before year-end when we close the books on 2022. It's been a great year, and I'm confident 2023 will be an even better one for JM kids everywhere.

Have a wonderful Holiday Season and New Year.

With appreciation,

Jim Minow
Executive Director, Cure JM Foundation
jamesminow@curejm.org