

**FOR IMMEDIATE RELEASE
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Cystinosis Ireland Announces Two Research Projects to be Funded Under HRCI-HRB Joint Funding Scheme 2022

Cystinosis Ireland is pleased to announce the funding of two research projects in partnership with HRB in the in the HRCI-HRB Joint Funding Scheme 2022

The scheme, which in this round had 34 applications from various health research funding Irish charities, is highly competitive with stringent review both by the submitting charity and by the HRB's processes. International peer review is standard with a firm focus on Public and Patient Involvement (PPI) where lay reviewers give input to the importance of the potential outcomes of the research.

Speaking about the importance of the funding for the projects for Cystinosis Ireland, Chair Mick Swift said:

"Cystinosis Ireland supports the best research we find internationally. The HRCI/HRB Joint Funding Scheme is of huge importance to our investment in research. It ensures our research funding processes meet the international standards required by the national health research funding body, the HRB, and it allows us to leverage our own fundraising to double its value. All of the fundraising undertaken by Cystinosis Ireland is invested in research. Being successful with two projects in this round of the scheme is a validation of the work we put into collaborating with the best researchers globally.

The Board of Cystinosis Ireland, and our community as a whole, offer our huge congratulations to Dr Hollywood, Professor Levtchenko, their collaborators and their teams on this achievement."

Speaking about her research project, which was ranked first of the 14 deemed fundable in the current round, Dr Jennifer Hollywood of Auckland University said:

"I'm delighted to have been awarded this funding in the current round of the HRCI/HRB Joint funding scheme. The work I will be doing is of great interest to the community and will build on previous work funded by Cystinosis Ireland through its Seedcorn grants. Working with Prof Herbie Newell, CBE, at the University of Sunderland to potentially develop an alternative therapy for cystinosis is very exciting. We are also very happy to have a strong PPI lead in Mr Will Newman of Cystinosis Foundation UK who will work with the research team on all aspects of the project."

Prof Elena Levtchenko of KU Leuven noted:

"Many people will have heard of the new technology of mRNA as a result of the vaccines delivered for Covid-19, and it is exciting to be able to apply these techniques to potentially improve the lives of those living with cystinosis."

Research - Awareness - Support

Cystinosis is a rare, degenerative, incurable disease that primarily affects children. It slowly destroys all the body's organs and muscles. Cystinosis Ireland is a volunteer-led, non-profit organisation dedicated to funding cystinosis research and providing support to those living with the condition.



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The Health Research Board (HRB) is a State Agency under the Department of Health, supporting and funding health and social care research. The HRB provides a stringent framework through which applications are made and a rigorous review process where only the best projects are approved for funding. Through the HRCI/HRB Joint Funding scheme 151 awards have been made to date, representing a total investment of €25 million. Each award is worth up to €300,000 for a project of between 12 and 36 months' duration and the partner charity provides 50% of the funding of the approved projects.

The Health Research Charities Ireland-Health Research Board's (HRCI-HRB) Joint Funding Scheme enables HRCI research charity members to co-fund with the HRB, research of particular interest to specific patient populations represented by those charities.

Research projects to be co-funded between Cystinosis Ireland and the HRB:

Title: Evaluation of a novel drug combination treatment for nephropathic cystinosis in a new cystinotic rat model.

Lead Applicant: Dr Jennifer Hollywood. **Host Institution:** Department of Molecular Medicine and Pathology, University of Auckland.

Co Applicants: Prof Alan Davidson, Dr Paddy Harrison and Mr Will Newman.

Collaborators: Prof Herbie Newell.

Duration: 2 years. **Total Funding Request:** €198,188. (Cystinosis Ireland total contribution €99,094).

Title: Investigating the potential of CTNS-mRNA loaded nanoparticles as a new therapeutic strategy for nephropathic cystinosis.

Lead Applicant: Prof Elena Levtchenko. **Host Institution:** University Hospitals Leuven & KU Leuven.

Co Applicants: Prof Bert van den Heuvel and Prof Roland Brock.

Collaborators: Dr Jenny van Asbeck-van der Wijst.

Duration: 3 years. **Total Funding Request:** €152,400 (Cystinosis Ireland total contribution €76,200).

Ends

For further information, please contact

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Directors: J. Ennis (NI), A. Maguire, S. Maguire, T. McDonald, L. McFadden, N. O'Brien, AM. O'Dowd, R. Reilly, M. Swift.

Patron: Stephen Rea

Charities Regulatory Authority Number: 20053796 Company Number: 371955



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Notes to the editor:

About Cystinosis Ireland

Cystinosis Ireland was founded in 2003 by those living with cystinosis and their families and continues to be led by people with personal experience of living with cystinosis. Cystinosis Ireland is one of the main patient organisations in Europe driving research into this rare disease. Since its establishment in 2003, Cystinosis Ireland has supported research projects focused on all aspects of this disease to the value of €2.6 million either through direct funding or as a co-funding partner.

About Cystinosis

Cystinosis is an extremely rare, genetic, metabolic disease with life-long effects. It is characterised by an accumulation of the amino acid cystine in organs and tissues of the body, leading to severe organ dysfunction. Cystine is stored in the lysosome of cells. In people with cystinosis, the transporter for cystine is dysfunctional, causing the cystine to build up in the cells and crystallise. The crystals quickly create toxic levels of cystine in the body, causing cellular dysfunction and even cell death. The soft tissues of the body and all organs are directly affected by this crystallisation, including the kidneys, eyes, liver, muscles, and central nervous system. At present there is no cure for cystinosis. Treatments are available which slow the progress of the disease but these can be difficult for patients to tolerate and may be required up to four times per day. Additional medications and supplements must be taken to replace the nutrients and electrolytes lost due to polyuria (the need to urinate very frequently). Specific medication is required in the form of eye drops to counteract the impact of the crystal build up in the corneas of the eyes.

Our vision: We strive for a cure for cystinosis. Until then, we want people with cystinosis to live the best life they can.

Our mission: We exist to raise awareness about cystinosis, support those affected by cystinosis, and invest in quality cystinosis research.

Our values: Empathy, determination, collaboration, innovation, integrity, professionalism

You can find more information about our work on www.cystinosis.ie

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