

### Spinal Muscular Atrophy Health Economics and Quality of Life Evaluation

The Newborn Screening Program Pilot for Spinal Muscular Atrophy (SMA) and Primary Immunodeficiency Disease (PID) team has recently published a paper on the health economic and health-related quality of life (HRQoL) burden on families with a child with SMA.

Published in 7<sup>th</sup> July 2020 edition of Neurology, (doi:10.1212/WNL.0000000000009715), the paper was given an editorial in the same edition (doi:10.1212/WNL.0000000000009716).

Lead by Professor Georgina Chambers of the NPESU, UNSW, the paper describes the direct and indirect cost associated with children with 5qSMA types I, II and III. The average total direct costs (vehicle and home modifications, outpatient care, and medical aids) was \$80,558 per year. Loss of income and unpaid informal care (indirect care) averaged \$63,146 per year.



**Professor Georgina Chambers**  
Lead Author  
Director, National Perinatal Epidemiological and Statistical Unit  
UNSW

Caregivers were surveyed on their HRQoL and the average score was 0.115 (0=death, 1=full health). The team remarked that the cost for caring for a child with SMA is not only an economic burden but also a burden on the quality of life.

The editors have commented that population based newborn screening programs is an opportunity to improve the quality of life of patients with SMA and their families.

### Innovation in Paediatric Precision Medicine Seed Funding

Expression of interest are invited for the Luminesce Alliance Innovation in Paediatric Precision Medicine Seed Funding. The intention of the seed funding is to get innovative research projects involving paediatric precision medicine off the ground, enabling them to seek further funding and/or assist with the translation into the clinical setting.

There is a cap of \$200,000 for each successful seed funding application with \$100,000 set aside to fund a health economics/health implementation paediatric precision medicine based project. The total funding pool is \$1,000,000.

Expression of interest will be open on the 15<sup>th</sup> September 2020 and close on the 30<sup>th</sup> October 2020. For further information, the terms and conditions of the seed funding round, please go to the Luminesce Alliance webpage link:

<http://www.luminesce.org.au/call-for-innovative-paediatric-precision-medicine-research-projects/>

or email:

[info@luminesce.org.au](mailto:info@luminesce.org.au)

Please note that only employees of one of the Luminesce Alliance partners are eligible to apply for the seed funding.

#### Seed Funding Important Dates

Open: 15<sup>th</sup> Sept 2020  
Closes: 30<sup>th</sup> Oct 2020  
Notified: 30<sup>th</sup> Nov 2020

## A/Prof Cheryl McCullagh Thank you

Luminesce Alliance would like to thank A/Prof Cheryl McCullagh, former Interim Chief Executive of the Sydney Children's Hospital Network (SCHN), for her significant contribution to the Alliance.

Her involvement on the Luminesce Alliance Board has helped shape and strengthen research direction of the Alliance. Luminesce Alliance wishes her well in her new role with NSW Health.

We also wish to welcome Cathryn Cox, who has been appointed the new Chief Executive of SCHN and to the Luminesce Alliance Board.



A/Professor Cheryl  
McCullagh  
Former Interim Chief  
Executive  
SCHN



Cathryn Cox  
Chief Executive  
SCHN

## Stronger Research Ties SCHN and CMRI

Luminesce Alliance partners, the Sydney Children's Hospital Network and the Children's Medical Research Institute have finalised an overarching umbrella agreement that will make it easier for researchers at both institutions to collaborate on biomedical research and their potential clinical applications.

It will simplify the documentation required for the expected increase in the number of collaborative research projects between the two partners.

Further information can be found at the <https://www.cmri.org.au/News/Latest-News/Sydney-Children-s-Hospital-Network-Formalises-Agre>

## 2019-2020 Luminesce Alliance Paediatric Precision Medicine Annual Report Thank you

A big thank you to everyone who has assisted in completing the activity workplans for their paediatric precision medicine program. The Executive Management Committee and the Board Chair have commended the progress achieved and documented in the report. The report has now been forwarded to the Office of Health and Medical Research NSW for their review.



### Our Partners



### Contact us:

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