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Re.: CIRM DISCO-18130 application

Dear CIRM ICOC ARS Members,

We are writing to express our appreciation of the insightful review of our CIRM DISCO Award application. We are excited that we received a fundable score as we are eager to start the proposed experiments. We believe that the results of these experiments will enable the development of a regenerative therapy for cholestatic liver diseases, most of which currently lack effective therapeutic options.

We would like to add that we are honored to represent the liver research field, with our application being the only one addressing liver disease. The specific disease focus of our application, primary sclerosing cholangitis (PSC), illustrates the medical need that exists for cholestatic liver diseases. Since no specific therapy is available, PSC progresses to end-stage liver disease and need for a liver transplant 15-20 years after diagnosis. Liver transplantation is in principle curative but the long-standing donor organ shortage means not every patient in need can be transplanted. Liver transplantation also has side effects, with many patients, particularly younger ones, describing it as trading one disease for another. And 25% of transplanted PSC patients are not stably cured because of graft failure caused by disease recurrence.

Our application harnesses a robust regenerative principle and innovative reprogramming technologies to address the medical need in PSC. Importantly, the results generated by our application will apply to other cholestatic liver diseases, with the broader medical impact being illustrated by cholestatic liver diseases accounting for 20% of adult and 50% of pediatric liver transplants in the US.

We thank you for your efforts supporting research leading to regenerative therapies for severe diseases.

Sincerely,

A stylized, handwritten signature in black ink, likely belonging to Holger Willenbring.

Holger Willenbring, MD, PhD  
Professor  
Liver Center  
Department of Surgery, Division of Transplant Surgery  
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A handwritten signature in black ink, likely belonging to Chris Hsiung.

Chris Hsiung, MD, PhD  
Assistant Professor  
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