20th SMA Researcher Meeting

https://neurodegenerationresearch.eu/survey/20th-sma-researcher-meeting/

Principal Investigators

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FAMILIES OF SPINAL MUSCULAR ATROPHY

Contact information of lead PI
Country

USA

Title of project or programme

20th SMA Researcher Meeting

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18348.62385

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1

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Research Abstract

Project Summary Spinal Muscular Atrophy (SMA) is an inherited neuromuscular disease that leads to motor dysfunction and death. It affects one in 6,000 to 10,000 babies born and is the leading genetic killer of children under two years of age. The molecular basis of the disease is a deficiency in production of a specific protein – Survival of Motor Neuron (SMN) protein. Motor neuron function is sensitive to lowered SMN protein levels, and this cellular defect leads to the loss of muscle function in the limbs, neck, and chest in these patients. Currently there is no treatment for SMA. Researchers have suggested that it is one of the neurological diseases closest to treatment, due to the unique presence of a back-up gene called SMN2 that provides

great therapeutic possibility. Cure SMA has sponsored the SMA Researcher Meeting for 20 years. In 2016, the conference will be held June 16 to 18 in Anaheim, California at the Disneyland Hotel. It is now the largest SMA research conference in the world with about 300 attendees each year. Basic researchers, clinicians, and industrial researchers all attend the conference, allowing for cross-disciplinary dialogue crucial to therapy development. The meeting is held simultaneously with the Annual SMA Conference, with 1,200 people affected by SMA attending, providing researchers a unique opportunity to interact with the patients they are dedicated to helping. The major goal of the meeting is to provide a venue for SMA experts worldwide to share unpublished data and develop scientific collaborations to hasten the identification of a treatment for SMA. Conference presentations are organized into 3 major areas: clinical research, basic research, and translational research. This allows researchers from different scientific disciplines to communicate about SMA. In addition, a major focus of the 2016 meeting will be a special session on “The Changing Landscape of SMA: Consideration of Future Needs”. The goal of the session is to discuss the implications for research, drug development, and clinical care as drug approvals possibly near. There will be an emphasis on how to move towards effective drug treatments and improved quality of life for all types of SMA and at all stages of disease progression. Therefore, this session will discuss approval of second-in-class drugs, the use of combination therapies, and the importance of registries and standards of care. In order to develop therapies to treat all SMA patients, it is critical for the scientific community to consider whether combination drugs or novel molecular approaches, beyond SMN regulation, should be pursued for the disease. Finally, another meeting goal is to introduce new scientists to SMA research. This includes researchers in training, helping to build the future of our research community and industrial researchers, helping to integrate them quickly into our research community.

Further information available at:

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