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## Dr. Elliot J. Androphy receives IGNITE grant to develop drug candidate for spinal muscular atrophy

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Dr. Elliot J. Androphy, the Kampen-Norins Professor and chair of the Department of Dermatology at the Indiana University School of Medicine, has been awarded an IGNITE, or Innovation Grants to Nurture Initial Translational Efforts, grant by the National Institutes of Health.

The grant will last three years, contingent upon achieving a set of predetermined milestones. Androphy has received \$431,954 for the first year of the grant. The funding will allow him and his colleagues to test compounds to determine the best candidate to treat spinal muscular atrophy, a genetic disease that affects voluntary muscle movement.

"The IGNITE funding covers tests to determine the compounds' toxicity, if they reach the blood-brain barrier, how quickly they are metabolized and other characteristics," he said. "My colleagues and I have done a lot of screening and validation, and a lot of the chemistry has been covered by other grants from the NIH. But we need to conduct these tests, which are very expensive."

Androphy said spinal muscular atrophy is caused by low levels of a protein called SMN, or Survival of Motor Neuron.

"Any treatments that increase SMN should mitigate the disease," he said. "Our goal is to increase SMN proteins in any number of ways. A significant part of the IGNITE grant will enable us to do this in mouse models."

The Indiana University Research and Technology Corp. has filed patent applications on Androphy's compounds.

"This intellectual property could lead to a therapy for spinal muscular atrophy, and in order to get a drug on the market, it needs to be patented and protected," Androphy said. "IURTC also is working with us to find a partner for some development work. Our hope is to take the best candidate to a clinical trial, which is an expensive step. Having a pharmaceutical company partner could move this forward as quickly as possible."

Androphy said receiving the IGNITE grant from the NIH has impacted him and his colleagues at Brigham and Women's Hospital/Harvard Medical School and the University of Missouri Medical School.

"This is the first round of funding for the national IGNITE program, and our grant was evaluated by six reviewers," he said. "The NIH review process is very rigorous, involving experts in drug therapies for neurological diseases. Knowing that we received the highest score of the session has strengthened our belief that we can impact people's lives by reducing the pain and suffering caused by spinal muscular atrophy."

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Source:  
Indiana University

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