

Wake Up Narcolepsy Impact Statement

The mission of Wake Up Narcolepsy is to increase awareness of narcolepsy, to educate the public and healthcare professionals about the signs and symptoms of narcolepsy and to support research towards improved treatments and a cure.

In line with the mission, we believe efforts in scientific research will lead to development of improved treatments as well as a cure for narcolepsy. Over the last several years we have funded basic and clinical researchers to further these efforts. We discuss some of them below. Our funds have led to novel findings of the genetic influence on susceptibility or protection, as well as brain regions involved with narcolepsy. Our funds have also facilitated the generation of important tools in the diagnosis of narcolepsy.

Wake Up Narcolepsy has funded Dr. Emmanuel Mignot of Stanford University who has made marked progress in identifying genetic markers of risk and protection from narcolepsy (Mignot [2013](#), [2015a](#), [2015b](#)). His research creates avenues to the development of diagnostic and predictive assays for the disease, as well as the potential for “immunization” against narcolepsy.

Wake Up Narcolepsy has funded Dr. Thomas Scammell of Harvard Medical School and BIDMC which led to the discovery that there is an increased number of neurons that release histamine in the brain of people with narcolepsy (Scammell [2013](#)). This novel observation led to investigation of whether a drug that targets the histamine system, Pitolisant, would promote wakefulness in people with narcolepsy. Since 2019 in the US, and 2016 in the EU, Pitolisant has been readily prescribed for narcolepsy with high efficacy in improving wakefulness and reducing cataplexy.

Cataplexy is ranked as one of the most disruptive symptoms of narcolepsy. We have funded multiple researchers who are pursuing the basic understanding of the neuronal circuits involved in the generation of cataplexy (Peever [2017](#) & Scammell [2017](#)). We have a better understanding of how emotions and social interactions may trigger cataplexy. These findings will inform the pursuit of pharmacological approaches to reduce cataplexy.

We have supported clinical researchers to better understand the impact of narcolepsy and the effectiveness of current treatments. In fact, the FDA-led patient focused drug development (PFDD) held in 2013 was the only of its kind to have a scientific paper (Maski [2017](#)). This was possible due to our support of Dr. Kiran Maski of Boston Children’s Hospital. Her research highlighted the need for better diagnostic tools for pediatric population and more educational resources to address a significant delay among patients with pediatric onset of symptoms. Her results showcased a broader range of symptoms that negatively impact and limit the activities in which they engage. Wake Up Narcolepsy has continued to fund clinical research aimed to develop novel markers of narcolepsy and idiopathic hypersomnia in children and to reduce the delay in diagnosis (Maski [2022](#), Malow [2017](#), Ong [2020a](#), [2020b](#), Narang [2018](#), [2019](#), [2022](#), Ingram [2021a](#), [2021b](#)).

"WUN aims to continue fundraising in order to accelerate narcolepsy research. More work needs to be done to understand how hypocretin cells are dying in the brain and whether an autoimmune process underlies the origin of narcolepsy for a majority of cases. Are there novel targets of pharmacological approaches to reduce symptom severity and lessen the impact narcolepsy has on activities of everyday life? Are the tools developed thus far for diagnosing narcolepsy, properly and widely used? These and other questions require our continued support and advocacy to achieve WUN’s mission" reports Monica Gow, Cofounder and Executive Director of Wake Up Narcolepsy.

The following sections are designed to highlight responses from the PIs and give some quick facts about the impact of WUN funding.

Statements from the PIs:

David Ingram: We have been very grateful for the support and services that WakeUp Narcolepsy provides. In terms of research support, their funding allowed us to perform a study of narcolepsy in children to help identify challenges, including psychosocial and medical comorbidities (PMID: 32895119), as well as, evaluate relative effectiveness of treatment approaches and care delivery for children with narcolepsy (PMID: 33283753). Beyond research, the organization provides important services for families and patients, such as education day events and support groups.

Quick reference to articles PMID: [32895119](#), [33283753](#)

Beth Malow: Thank you again for your generosity in wanting to support our research on narcolepsy. We are excited about partnering with your foundation!

PMID: [29117887](#)

Kiran Maski: Our research group endeavors to improve timely diagnosis of pediatric narcolepsy. With WUN support, we developed and validated the Pediatric Hypersomnolence Survey now published in the journal Neurology <https://pubmed.ncbi.nlm.nih.gov/35314496/>. We hope this survey can be used in health care settings and the community to more quickly identify children and adolescents with potential narcolepsy or idiopathic hypersomnia for more directed expert referrals and appropriate diagnostic testing.

PMID: [27923434](#), [35314496](#)

Emmanuel Mignot: Dr. Emmanuel Mignot and his research team have completed Genome Wide Association studies to uncover why narcolepsy develops in some individuals but not others. This includes populations that received the Pandemrix vaccine. Additionally, the research team generated a computer program to screen more patients in a more efficient manner than the MSLT by identifying 5 predictive markers of narcolepsy.

PMID: [24204295](#), [25452148](#), [25574827](#), [26299470](#), [26611950](#), [30523329](#)

Indra Narang: The Money from WUN has been used to conduct both qualitative and quantitative research in Toronto and more specifically, the money has been/is used to pay for research personnel to conduct the research. The money is also being used for a collaborative research project with Dr. Kiran Maski at Boston Children's hospital.

PMID: [30455579](#), [31070768](#), [31844391](#), [32651734](#), [33877929](#)

Ariel Neikrug: Dr. Neikrug recently received funds from WUN and aims to address how the behavioral pattern of day-to-day life is impacted by narcolepsy. Dr. Neikrug wrote "With the money from Wake-Up Narcolepsy we will be able to utilize existing data for Behavioral activity rhythms characterization and to set the stage and yield preliminary data that will then support future NIH-funded research."

Jason Ong: We greatly appreciate the support from WUN, which has allowed us to carry out and complete these projects during the pandemic. We hope that our work will provide evidence-based strategies using digital and tele-health delivery to improve the mental health and quality of life among people with narcolepsy.

PMID: [31937147](#), [32804069](#)

John Peever: WUN funding has allowed me to support, in part, 2 PhD students whose research is focused on mechanisms of cataplexy in narcolepsy. WUN funding has allowed us to published 2 papers (Current Biology and The Neuroscientist).

PMID: [28209737](#), [31679942](#), [34704497](#)

Tom Scammell: Our research group focuses on understanding the brain mechanisms of narcolepsy, from the roles of specific types of neurons to how these cells work together to produce sleepiness and cataplexy. Funding from WUN has supported research on how loss of the orexin/hypocretin neurons in people with narcolepsy can increase the number of neurons making histamine. Using mouse models of narcolepsy, the lab has also studied the functions of these histamine-producing neurons; how the age of narcolepsy onset may relate to severity of symptoms; the role of the amygdala in emotion-triggered cataplexy; and how oxytocin contributes to the occurrence of cataplexy in social circumstances. This flexible funding from WUN enabled Dr. Scammell to obtain pilot data that was instrumental in obtaining large NIH research grants and complete high impact experiments.

PMID: [23904672](#), [24006291](#), [28235898](#), [32249922](#), [33746708](#)

Wake Up Narcolepsy Bullet points

Total amount of money funding research since 2009: \$1,064,706.32

of researchers funded: 9

Institutions represented:

Beth Israel Deaconess Medical Center and Harvard Medical School
Boston Children's Hospital and Harvard Medical School
Northwestern University/Feinberg School of Medicine
Stanford University/ Stanford Center for Sleep Sciences and Medicine
University of California-Irvine
University of Missouri-Kansas City
University of Toronto
Vanderbilt University Medical Center

of publications acknowledging WUN or with confirmation funds were used to support the research: 26

Impact Factor by journal WUN funded researchers have published in:

19.23 Brain, Behavior, Immunity (Mignot)
17.69 Nature Communications (Mignot)
10.83 Current Biology (Peever)
10.5 American Journal Human Genetics (Mignot)
10.42 Annals of Neurology (Scammell)
9.901 Neurology (by American Academy of Neurology) (Maski)
7.519 The Neuroscientist (Peever)
6.167 The Journal of Neuroscience (Peever, Scammell)
5.917 PLoS Genetics (Mignot)
5.85 Sleep (Narang, Scammell (2))
5.061 Current Neurol Neuroscience Rep (Narang (2))

- 3.586 Journal of Clinical Sleep Medicine (Maski, Malow (1), Ingram (2), Ong)
- 3.568 Sleep Medicine (Mignot (2))
- 3.566 Frontiers in Neuroscience (Scammell)
- 3.171 Behavioral Sleep Medicine (Ong, Narang)
- 1.979 Child Health (Narang)

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