This newsletter is provided through the generosity of the Doris and Donald Schnuck Fund for Children in Need and the St. Louis Children’s Hospital Foundation.

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The event included games, bounce houses, music, a live auction, silent auction bidding, and the infamous Cow Patty Bingo. This year’s FunFest raised an impressive $27,574.64, which will fund Gutmann Laboratory research initiatives.

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- NF Center Director, David H. Gutmann, MD, PhD

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WE'VE BEEN ABLE TO SCREEN CHILDREN AT OUR CENTER, IDENTIFY AUTISM SPECTRUM DISORDER, ATTENTION-DEFICIT DISORDER AND PROBLEMS WITH EXECUTIVE COGNITIVE FUNCTION...AND ENABLE THESE CHILDREN TO GET THE RESOURCES AND SUPPORT THEY NEED - SPECIFICALLY ACADEMIC AND SOCIAL SUPPORT – TO IMPROVE THEIR QUALITY OF LIFE.

- First author, Stephanie M. Morris, MD

SCIENTISTS LINK SINGLE GENE TO SOME CASES OF AUTISM SPECTRUM DISORDER

This article, written by Jim Dryden, originally appeared in the Washington University School of Medicine News Hub on October 19, 2016.

Scientists have linked mutations in a single gene to autism in people who have a rare tumor syndrome typical of patients diagnosed in childhood. The findings, in the journal JAMA Psychiatry, may lead to a better understanding of the genetic roots of autism in the wider population. The findings are published Oct. 19 in the journal JAMA Psychiatry.

Studying 581 patients at six clinical centers in the United States, Belgium, the United Kingdom and Australia, the researchers found that mutations in the NF1 gene that cause the disease also contributed to autistic behaviors in almost half of the patients. “NF1 is caused by mutations in a single gene — NF1,” said first author Stephanie M. Morris, MD, an instructor in neurology. “Our research indicates that this single gene also is associated with autism spectrum disorders in these same patients. That makes it possible to look downstream from the gene to find common pathways that contribute to autism in the wider population.”

NF1, the disorder caused by NF1 mutations, usually appears during childhood. Symptoms can vary in severity, but they include café-au-lait spots, which are flat, brown spots on the skin. Other symptoms include tiny nodules on the iris of the eye, tumors, bone deformities such as a curved spine or a bowed leg, and optic gliomas, tumors of the optic nerve. Kids with NF1 also can have learning disabilities.

“In the 25-plus years that I’ve taken care of kids with NF1, I’ve often noted that a small percentage of these kids have what I would term autism traits,” said senior investigator David H. Gutmann, MD, PhD, the Donald O. Schnuck Family Professor of Neurology and director of the Washington University School of Medicine’s Neurofibromatosis Center.

Gutmann and his colleagues have been working to identify which particular misfiring genes may interact along that same pathway to contribute to autism in people who don’t have NF1. Until now, they recently started to recognize that these children also often have symptoms of autism.”

About 100,000 people in the United States have NF1. It is equally common in both sexes and among all racial groups. Autism, meanwhile, affects 1 percent to 2 percent of all children in the United States and is four to five times more common in boys than in girls.

“What’s unique about our findings is that it’s likely mutations in the NF1 gene are driving most of the symptoms of autism in children with NF1,” said the study’s other senior investigator, John N. Constantino, PhD, the Blanche F. Ittelson Professor of Psychiatry and Pediatrics and director of the Wilkens Greenleaf Elliott Division of Child & Adolescent Psychiatry. “Here, we have a single-gene disorder that affects a fairly large number of people and is causing autism in a significant number of those who are affected. This work could provide us with an opportunity to study a single gene and figure out what it is doing to cause autistic syndromes.”

Constantino said most autism spectrum disorders are influenced by multiple genes but that isolating this one gene can aid efforts to learn how other, unrelated genes may interact along that same pathway to contribute to autism in people who don’t have NF1. Until now, they were unsure how those various genes come together to cause symptoms eventually could lead to better treatments. But already the findings are benefiting children and families treated at the Washington University NF Center.

“We’ve been able to screen children at our center, identify autism spectrum disorder, attention-deficit disorder and problems with executive cognitive function.” Morris said. “And when we identify these deficits in kids, we can tell their parents, inform their schools and enable these children to get the resources and support they need - to enjoy academic and social support – to improve their quality of life.”


This work was supported by the Fusco Kennedy Driver Institute of Child Health & Human Development of the National Institutes of Health, grant number HD097571. Additional funds came from Schnuck Markets, Inc., the Neuronal Sciences Academic Development Award at Washington University School of Medicine, the NIH New Innovator Award 1DP2OD007483 and the Emerging the Future grant of KU Leuven.
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Scientists have linked mutations in a single gene to autism in people who have a rare tumor syndrome type 1 (NF1) and are more likely to understand how mutations in a single gene can contribute to symptoms of autism, such as problems with social and language skills and repetitive behaviors.

About 100,000 people in the United States have NF1. It is equally common in both sexes and in all racial groups. Autism, meanwhile, affects 1 percent to 2 percent of all children in the United States and is four to five times more common in boys than in girls.

"What’s unique about our findings is that it’s likely mutations in the NF1 gene are driving most of the symptoms of autism in children with NF1," said the study’s senior investigator, John N. Constantinou, the Blanche F. Itelson, Professor of Psychiatry and Pediatrics and director of the Wilkens Greensfelder Elliott Division of Child & Adolescent Psychiatry. “Here, we have a single-gene disorder that affects a fairly large number of people and is causing autism in a significant number of those who are affected. This work could provide us with an opportunity to study a single gene and figure out what it is doing to cause autistic syndromes.”

Constantino said most autism spectrum disorders are influenced by multiple genes but that isolating this one gene can aid efforts to learn how other, unrelated genes may interact along the same pathway to contribute to autism in people who don’t have NF1. He recently started to recognize that these children also often have symptoms of autism," said senior investigator David H. Gutmann, MD, PhD, the Donald O. Schnuck Family Professor of Neurology and director of the Washington University NF Center. "In the past, we didn’t really understand the association between NF1 and autism, but now we have new insights into the problem, which will allow us to design better treatments for children with NF1 and autism.”

The findings also could help scientists who study the genetics of autism understand how mutations in a single gene can contribute to symptoms of autism, such as problems with social and language skills and repetitive behaviors.

YUAN PAN, PhD

received a MCDONNELL FOUNDATION FELLOWSHIP to study the role of immune system cells in malignant brain tumor growth. These studies are aimed at developing new therapies for these deadly brain cancers.

MATTHEW STROH, PHD

received a HEMATOLOGY T32 FELLOWSHIP to better understand how the NF1 gene controls cell growth under different conditions, with the hope of discovering how this important gene functions as a tumor suppressor in the brain.

ANGELA HIRBE, MD, PHD

received a ST. LOUIS MEN’S GROUP AGAINST CANCER GRANT and SARCOMA ALLIANCE CAREER DEVELOPMENT GRANT to launch her independent career as a physician-scientist, and to continue her research on a deadly cancer seen in young adults with NF1. She recently joined the faculty at Washington University where she will direct a research laboratory focused on developing new treatments for malignant peripheral nerve sheath tumors (MPNSTs) arising in people with NF1.

The Washington University Neurofibromatosis (NF) Center enable our mission to find better treatments for children and adults with neurofibromatosis (NF). Washington University is a federal tax-exempt organization. Learn more about supporting the NF Center at NFCENTER.WUSTL.EDU/GIVE.

GREAT WAYS TO SUPPORT THE WASHINGTON UNIVERSITY NF CENTER

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1 Visit GIFTS.WUSTLEDU, select “NF Center” in the Centers and Institutes drop-down menu and complete the form to submit a monetary donation to support research at the Washington University NF Center. Financial contributions may be provided as an endowment, bequest, gift, or tribute to a loved one with neurofibromatosis.

2 Register to receive a FREE Schnucks eScrip Community Card, have it scanned each time you shop at Schnucks Markets and Schnucks will donate a percentage of your grocery bill to the Washington University NF Center! Just by shopping at Schnucks and scanning your community card, you are supporting the Washington University NF Center! Sign up for your eScrip card at NFCENTER.WUSTLEDU/GIVE/SHOP-FOR-NF/SCHNUCKS-ESCRIP.

3 The Washington University NF Center has partnered with Bravelets to help families and individuals affected by NF. With each Bravelets item purchased, $10 is donated to the Washington University NF Center. There are a variety of Bravelets items to choose from including bracelets, necklaces and rings for both men and women. They make excellent holiday and birthday gifts, and serve as a reminder to be brave throughout all of life’s challenges. Shop for Bravelets at BRAVELETS.COM.
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