## Innovative Gene Therapy 'Reprograms' Cells to Reverse Neurological **Deficiencies**

Surgery that produced unprecedented improvements in children with rare genetic disorder may also treat Parkinson's, Alzheimer's disease

\*Note: Embargoed until Monday July 12th, 2021 at 5:00 a.m. eastern

The Ohio State University Wexner Medical Center

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| NEWS PACKAGE  |   |
|---|---|
| SUGGESTED TEASE   | A NEW APPROACH TO GENE THERAPY OFFERS HOPE TO THOSE LIVING WITH INCURABLE GENETIC DISORDERS  COMING UP, HOW "REPROGRAMMING" BRAIN CELLS IS CHANGING LIVES.  |
| (PACKAGE START)   | THERE ARE HUNDREDS OF NEUROGENETIC DISORDERS IN WHICH THE MUTATION OF ONE SINGLE GENE CAN CAUSE A LIFETIME OF DISABILITY FOR THOSE WHO INHERIT THEM. AND WHILE MOST OF THESE CONDITIONS HAVE NO CURE OR EVEN EFFECTIVE TREATMENTS, A NEW STUDY OFFERS HOPE FOR SIGNIFICANT IMPROVEMENT BY "REPROGRAMMING" CELLS IN THE BRAIN TO REVERSE NEUROLOGICAL DEFECTS. BARB CONSIGLIO HAS THE DETAILS ON HOW THIS NEW PROCEDURE IS CHANGING THE LIVES OF CHILDREN BORN WITH A RARE BUT SEVERE GENETIC DISEASE. |
| CG: Courtesy: The Ohio State University Wexner Medical Center :00 - :03 | (Nats - Sound) :02  AS AN INFANT, JAMELL STAGG JUNIOR WASN'T  |
| Shots of Stagg family   | HITTING NORMAL DEVELOPMENTAL MILESTONES. :04  |
| CG: Shante Stagg<br>Child has rare genetic disorder                     | "At the three-month mark his pediatrician is actually the one who thought something was wrong." :05   |
| Shots of Stagg family   | JAMELL WAS EVENTUALLY DIAGNOSED WITH A RARE GENETIC DISORDER THAT CAUSES HIM TO BE DEFICIENT IN AN ENZYME CALLED A-A-D-C, WHICH IS RESPONSIBLE FOR MAKING DOPAMINE IN THE CENTRAL NERVOUS SYSTEM. DOPAMINE FUELS TWO IMPORTANT PATHWAYS IN  |
| Animation of brain  | THE BRAIN, ONE THAT IS RESPONSIBLE FOR MOTOR  |

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FUNCTION AND ANOTHER THAT REGULATES MOOD AND EMOTION. WITHOUT IT. CHILDREN BORN WITH THIS DISORDER SUFFER SEVERE PHYSICAL AND Shots of Stagg family DEVELOPMENTAL DISABILITIES AND REQUIRE ROUND-THE-CLOCK CARE. :23 CG: Dr. Brad Elder "They sleep poorly, they have what are called **Ohio State Wexner Medical Center** oculogyric crises, which are akin to seizures. They have very poor motor control, very poor motor movements.":11 BUT A NEW STUDY OFFERS HOPE FOR THESE Shots of researchers in lab PATIENTS AND POTENTIALLY FOR MANY OTHERS WITH GENETIC AND NEURODEGENERATIVE DISEASES. RESEARCHERS AT THE OHIO STATE UNIVERSITY Shots of gene therapy surgery WEXNER MEDICAL CENTER SUCCESSFULLY PERFORMED A GROUNDBREAKING GENE THERAPY SURGERY, INFUSING A BENIGN VIRUS PROGRAMMED WITH SPECIFIC D-N-A INTO Timelapse of MRI imaging during TARGETED AREAS OF THE BRAIN, DELIVERED surgery SLOWLY AS DOCTORS MONITOR ITS PROGRESS THROUGH REAL TIME M-R-I IMAGING. :22 "Really what you're doing is you're introducing to the Dr. Elder (CG'd earlier) cell, a plan, a different code.":07 IN THE STUDY. THOSE PAINFUL. SLEEPLESS Shots of gene therapy surgery NIGHTS THAT PLAGUE KIDS WITH A-A-D-C DEFICIENCY, DISAPPEAR. :07 "This is the first thing we see that goes away CG: Dr. Krystof Bankiewicz completely, remarkably within a few weeks of the gene Ohio State Wexner Medical Center therapy. It never comes back.":07 AND IN THE MONTHS THAT FOLLOWED. SEVERAL PATIENTS WERE ABLE TO SIT UP, FEED Video of study participants' THEMSELVES, OR EVEN WALK - UNPRECEDENTED milestones IMPROVEMENTS AMONG CHILDREN WITH THIS DISORDER.:07 (nats of family):01 FOR JAMELL, IT COULD MEAN A FUTURE HIS Shots of Stagg family PARENTS HAVE DREAMED FOR HIM.: 05 CG: Jamell Stagg "Just to be able to see him actually pick up a toy or Child has rare genetic disorder something, is going to be nothing but joy.":05 AT THE OHIO STATE WEXNER MEDICAL CENTER, Shots of Stagg family THIS IS BARB CONSIGLIO REPORTING: :03 (PACKAGE END) -----RESEARCHERS BELIEVE THIS SAME METHOD OF ANCHOR TAG TREATING KIDS WITH A-A-D-C DEFICIENCY CAN BE

USED TO TREAT OTHER GENETIC DISORDERS AS WELL AS COMMON NEURODEGENERATIVE DISEASES LIKE PARKINSON'S AND ALZHEIMER'S DISEASE.

CLINICAL TRIALS ARE CURRENTLY UNDERWAY TO TEST THIS TYPE GENE THERAPY IN OTHERS LIVING WITH DEBILITATING AND INCURABLE NEUROLOGICAL CONDITIONS.

#### **SOCIAL MEDIA**

Share it! Suggested tweet:

A new gene therapy procedure offers hope for those with genetic and neurodegenerative diseases. In a new study, researchers at <u>@OSUWexMed</u> found the surgery resulted in unprecedented improvements in children born with a rare genetic disorder. <a href="https://bit.ly/3m2y78m">https://bit.ly/3m2y78m</a>.

Suggested post:

For those living with genetic and neurodegenerative diseases, there are often few treatment options. But an innovative gene therapy pioneered by researchers at <a href="The Ohio State University Wexner Medical Center">The Ohio State University Wexner Medical Center</a> offers new hope. The study found that the surgery that "reprograms" cells in the brain resulted in unprecedented improvements in children born with a rare genetic disorder and may be expanded to treat conditions such as Parkinson's and Alzheimer's Disease. <a href="https://bit.ly/3m2y78m">https://bit.ly/3m2y78m</a>.

#### **EXTRA BITES**

Bankiewicz describes the improvements they've seen over time:

"Many of the subjects that are close to three years right now, they go back to school, they are able to walk. Some of them started speaking. So it's just a matter of time for them to catch up on all the lost time that they lost, in terms of their abnormal development.":17

CG: Dr. Krystof Bankiewicz
Ohio State Wexner Medical Center

Bankiewicz says this method is being tested for conditions such as Alzheimer's Disease:

"We're learning from what we're doing in this particular trial. And we get a lot of ideas how to do other conditions, such as Alzheimer's disease. This is a trial that we'll start very soon, which is based exactly on the same delivery method that's so good if it works in these children." :20

CG: Dr. Brad Elder Ohio State Wexner Medical Center Elder says MRI provides important information in real time: "The MRI is really used when you need either very fine detail or you need a live assessment, you need to know what is happening right the second you can't find out hours later by finishing a surgery and waking the patient up and then doing an MRI":17

Elder says kids who have this procedure keep improving over

# CG: Dr. Brad Elder Ohio State Wexner Medical Center

time:

"Over a long period of time, one year, two year, three years, some of these children have not only regained the ability to just sit or sleep through the night or stop having oculogyric crisis, which all the kids have made those accomplishments, but to be able to walk and feed themselves." :21

Elder explains why precise MRI mapping is so important: "Whichever area of the brain you're targeting, you have to have a clean pathway to get there. And there's structures, of course, that are pretty common sense that you can't violate. And blood vessels are a big one, because that would cause bleeding and the ventricles.":14

Shante says seeing her son do normal things will be amazing: "We get to see kids his age doing, typically children doing typical things and it's like, if only he could do those things. And I know that when he gets to, and if he gets to, it's just going to... I don't know, I don't feel like I have a word to explain how I would feel." :20

CG: Shante Stagg Child has rare genetic disorder

Shante explains how they finally got an accurate diagnosis: "His neurologist actually one day was like, 'You know, I've been researching. I think I know what this is. It's super rare. There's only 130 kids in the entire world that have it. So we're going to test him for it.' And it was an easy test. It was a blood draw and that's what it came out to be.":16

Jamell says the surgery went well and hopes are high: "Surgery went really smooth and they got good coverage in the two spots that they were targeting and they believe that they have high hopes for him." :11

CG: Jamell Stagg Child has rare genetic disorder

Jamell says he leaned on his faith as his child had surgery: "I just put all my faith in God and just kind of prayed and had my church group pray, had my close family members pray and me and her shed tears and we're just like, we're handing our child over not only to a doctor, but it's in God's hands and we kind of just leaned on that.":18

#### References

<sup>1</sup>Gene therapy for aromatic L-amino acid decarboxylase deficiency by MR-guided direct delivery of AAV2-AADC to midbrain dopaminergic neurons, Nature Communications, 12th July 2021.

Online: <a href="http://dx.doi.org/">http://dx.doi.org/</a>

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