

Gene Therapy and Immunotherapy Approaches for Neurofibromatosis Type 2

NF2 BioSolutions (NBS) is currently accelerating and supporting ten research laboratories from world renowned institutions which includes **distinct gene therapy approaches** and a **unique bacteriotherapy approach** for the **treatment of Neurofibromatosis Type 2**.

BACTERIOTHERAPY APPROACH

HARVARD MEDICAL SCHOOL
Harvard/MGH

Gary J. Brenner MD, PhD

John J. Mekalanos, PhD

Tumor shrinkage in **NF2 schwannoma mice** treated with engineered bacteria

Bacteria re-engineered for optimization

The Mekalanos lab, in collaboration with the Brenner lab, is working with **genetically-modified bacteria** to kill NF2 tumor cells through **direct killing, inducing anti-tumor immune response, and inhibiting blood vessel formation.**

Licensed to

MULBERRY BIOTHERAPEUTICS

Mulberry Biotherapeutics

- Designing **human clinical trials**
- Raising **Angel investors funds**

Links

[Dr. Brenner & Dr. Mekalanos' research webinar](#)

[Mulberry 2023 webinar updates](#)

[Bacteriotherapy approach scientific paper](#)

SUICIDE GENE APPROACH

HARVARD MEDICAL SCHOOL
Harvard/MGH

Gary J. Brenner MD, PhD

The Brenner lab is developing a **cytotoxic gene therapy strategy** to insert a "suicide gene" **ASC** that targets and kills schwannoma cells to **shrink existing tumors.**



Creation of a **suicide gene construct** expressing the **ASC gene**. Tumor shrinkage in **NF2 schwannoma mice** treated with viral ASC gene delivery.

Testing the possibility to deliver the ASC gene through a modified bacteria

GENE ADDITION APPROACH

NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.
Nationwide Children

Kathrin C. Meyer, PhD

The Meyer lab is investigating an **NF2 gene addition approach** to insert the **functioning NF2 gene** into mutant cells and **halt tumor growth.**



Creation of **several AAV constructs** expressing Merlin protein

Testing of AAV viral constructs in **NF2 Xenograft mice**

HARVARD MEDICAL SCHOOL
Gary Brenner, MD, PhD

Pre-IND

First discussion with FDA for trial design planning

NF2 BioSolutions/ Merlin is applying for a NIH grant

GENE REPLACEMENT APPROACH

University of Massachusetts
UMASS Medical School
UMass

Terence R. Flotte MD

The Flotte lab will be using an **NF2 gene replacement strategy** to **silence** the mutated NF2 gene and **insert** the functioning NF2 gene into defective cells to **halt tumor growth.**



Creation of **several AAV constructs** to **silence** the mutated NF2 gene and **insert** a functioning copy

Testing of AAV viral constructs in **NF2 knockout mice**

INDIANA UNIVERSITY SCHOOL OF MEDICINE

D. Wade Clapp, MD

LEGEND

- Funded by NBS
- Supported by NBS
- Done
- Ongoing
- In planning

Tools for Researchers to study Neurofibromatosis Type 2

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