

# Biologics

Pioneers Deliver on the Promise of Nature's Medicine Chest

Microscopic image shows a mouse cochlea treated with a viral vector developed by Dr. Jeffrey Holt (right).

## There's a revolution underway in medicine.

Scientists are turning to nature and engineering new treatments—biologics—to mimic the intricacies of life itself.

But what are they, exactly?

Unlike conventional chemically synthesized medicines, biologics are derived directly from natural molecules and offer precise disease targeting and a potentially lower risk of unforeseen side effects.

While big pharmaceutical companies excel in later-stage clinical and manufacturing development, biologics require the dedication and tenacity during early discovery found in academic labs, a sweet spot for Boston Children's. "It's a unique strength for us," says Executive Vice President and Chief Scientific Officer **Nancy Andrews, MD, PhD**, "as these innovations can evolve from our laboratories to clinical practice and, eventually, to commercialization."

Meet three scientists harnessing the building blocks of biology to unlock groundbreaking interventions.



### Revolutionizing Treatments for Genetic Hearing Loss

For the millions of patients affected by hearing loss and deafness, the only available treatments are devices like hearing aids or cochlear implants. While these interventions can be beneficial, they don't work in every case and fall short of replicating natural hearing.

**Jeffrey Holt, PhD**, and his team have explored multiple gene therapy approaches and demonstrated they can restore hearing in mouse models of three forms of human genetic hearing loss. What

sets their efforts apart is the seamless interaction between laboratory research and clinical practice. They're engineering mice with genetic mutations similar to humans and generating patient-derived stem cells to grow inner ear tissue, an approach that offers valuable tools for controlled testing and validating potential treatments. Thanks to essential support from donors like **Jeffrey Barber** and **Kimberly Hsu-Barber**, Dr. Holt's work could transform the landscape of genetic hearing loss therapies, offering new possibilities for patients to experience a world filled with sound.