From Scientific Discovery to Real World Application: the "Future" of Medicine is Here

I vividly remember the day in February of 2001 when the nearly complete sequence of the human genome was first published. My cellular and molecular biology professor had unfolded a giant poster from the journal Nature and revealed those approximately 30,000 genes to our class. Amazingly, in less than two decades since that seminal moment in science, we've seen the development of several new classes of medicine designed to treat diseases caused by specific gene mutations, as well as genetic approaches to treat some types of cancer. My colleagues and I are both excited and humbled to be a part of this incredible movement in science as we help bring revolutionary therapeutic approaches based on remarkable discoveries to market.

Gene Therapy

The clinical application of molecular biology that has always most intrigued me is the potential to cure a genetic disease by supplying someone with a "healthy" copy of his or her dysfunctional gene. Unfortunately, there were many early gene therapy failures, including the tragic death of a healthy teenage clinical trial participant in 1999. After decades of disappointment, the promise of gene therapy was finally realized in 2017 with the FDA approval of two distinct gene therapy approaches.

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The first gene therapy for an inherited disease was approved for a form of hereditary blindness. This therapy, which delivers a healthy gene into the cells of the eye via a modified virus, offers a life-changing potential for affected individuals.

Chimeric antigen receptor (CAR) T-cell therapy brought gene therapy to oncology. With CAR T-cell therapy, a patient's own immune cells are isolated and genetically engineered to make a specific receptor that allows the modified cells to recognize and attack cancer cells when introduced into the body. Two CAR T therapies have been approved by the FDA and are offering renewed hope for people with certain types of leukemia and lymphoma.

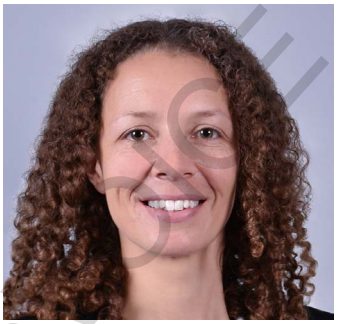
RNAi Therapeutics

2018 brought the FDA approval of the first RNAi therapeutic. RNA interference, or RNAi, is a process that our cells use to regulate the expression of genes. The pathway was discovered in the 1990s through work in nematodes and plants, and the scientists behind the discovery of RNAi won the Nobel Prize in Physiology or Medicine in 2006. A little more than a decade later, this groundbreaking science, which can be used to disrupt production of disease-causing proteins, has made its way to families with a progressive and debilitating rare disease. This turning point has the potential to change the lives of people living with rare genetic conditions today, as well as create a new future for generations of families with hereditary conditions.

Genome Editing

Perhaps the next big advancement in medicine will also come from a basic science discovery, this time with bacteria. Genome editing refers to the process of changing an organism's DNA. While multiple approaches exist, the CRISPR-Cas9 (clustered regularly interspaced short palindromic repeats and CRISPR-associated protein 9) approach is the one generating the most attention. CRISPR-Cas9 harnesses an immune mechanism from bacteria to target a specific DNA sequence to be cut and altered by adding, removing, or changing the DNA sequence. If proven safe and effective in humans, this approach could potentially be applied broadly to treat many genetic diseases. The first genome editing clinical trials in the United States and European Union were initiated in 2018. Genome editing is currently being explored as a therapeutic approach to alter the immune cells of patients with multiple myeloma, melanoma, or certain types of soft tissue sarcoma to recognize and destroy cancer cells.

While my professional career has taken me from basic science to healthcare marketing, my enthusiasm about these incredible scientific discoveries and what they mean for patients has only increased. The rare disease space continues to transform our approach to healthcare; instead of thinking solely about medicines to treat symptoms, we may look at genes as the root cause of these symptoms, especially knowing mutations now have the potential to be addressed with targeted therapies. My colleagues and I consider ourselves incredibly fortunate to be a part of this monumental shift, and I personally can't wait to see what else the future holds for genomic medicine. PV



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WHY UNIFY

In specialty and rare disease communities, HCPs do not always see the totality of a patient's disease burden or the benefits that new therapies may provide. Likewise, patients often struggle to express their needs or be their own advocates for treatment.

It is our belief that only by uniting these audiences—through empathy, understanding, and shared responsibility—can we help specialty brands reach their true potential.

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