



## **Pioneer in RNA-targeted therapeutics to collaborate with academia and think tanks to identify next-wave technologies with potential to pioneer new markets in healthcare**

### **Ionis launches ‘Ion-ARPA’ initiative with an eye toward revolutionizing drug discovery – again**

The field of antisense technology was born in 1978, when antisense RNA was first used to inhibit protein expression. In 1989, Ionis was founded to make drugs out of antisense oligonucleotides: short, single-stranded RNAs that bind to messenger RNA to control protein production. It was a revolutionary (some said ‘crazy’) idea that, at the time, did not have much support outside the walls of Ionis’ labs near San Diego, Calif. Today, Ionis is recognized as the pioneer of RNA-targeted therapeutics, with three marketed medicines and a premier late-stage pipeline highlighted by industry-leading neurological and cardiometabolic franchises.

Unsatisfied to rest on its laurels, Ionis is now planting seeds to continue to innovate by launching Ion-ARPA, a new initiative designed to identify and accelerate the creation of therapeutic technologies through disruptive innovation. Modeled on the U.S. Department of Defense program known as DARPA (Defense Advanced Research Projects Agency), the Ion-ARPA approach will contribute to the company’s long-term strategy by expanding on the innovation that brought about its revolutionary antisense technology to eventually create novel cutting-edge technologies capable of pioneering new markets in healthcare.

“Ion-ARPA programs will be funded based on big high-risk ideas with big payoff potential”, said Ionis co-founder Dr. David Ecker, who conceived and leads the Ion-ARPA initiative. “Programs are intended to run for two years to test bold ideas, with the potential to continue if encouraging results are obtained.” Dr. Ecker has an intimate understanding of the DARPA process, having been a DARPA performer himself for many years leading projects that resulted in the first industry effort to target structured regions of RNA with small molecules and new biosensor technology.

#### **KEY TAKEAWAY**

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The first Ion-ARPA program will explore quantum mechanics, its role in biology and potential impact on disease processes. The program is being conducted in partnership with the University of California at Santa Barbara, the University of California Riverside, and the University of Buckingham, in the UK.

“Among our first areas of focus is the hypothesis that biological molecules may store information in a quantum fashion, essentially using the same principles as a quantum computer. This has implications for our understanding of brain functionality, with potential applications for neurological and psychiatric illnesses,” explained Dr. Ecker who is also vice president of Strategic Innovation at Ionis.

“Truly disruptive new ideas often emerge from the cross pollination of different disciplines. While the disciplines of chemistry and biology have long been merged almost into a single discipline, physics and biology have not blended to nearly the same extent. That collaboration is long overdue,” said Dr. Ecker. “There is a revolution about to happen in science as physicists begin to engage collaboratively with biologists. That will lead to fundamental new understandings of disease processes and point the way to new therapeutic strategies that are not on anyone’s radar today.”

Ionis also intends to expand the Ion-ARPA approach into other exciting new areas that are ripe for the discovery of novel therapies for human diseases that keep the company on the cutting edge of innovation. This includes diversification of drug discovery capabilities, moving Ionis closer to achieving the goal laid out by its CEO Brett P. Monia, Ph.D., of improving the lives of millions of patients around the world.

Learn more about these collaborations at:

<https://www.ionispharma.com/ionis-innovation/ion-arpa-initiative/>