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THE PAUL G. ALLEN FRONTIERS GROUP NAMES UC BERKELEY'S JENNIFER DOUDNA A NEW ALLEN DISTINGUISHED INVESTIGATOR

Pioneer of gene editing will explore new territory of RNA targeting and more complex cell editing

WASHINGTON, D.C. — March 23, 2016 — The Paul G. Allen Frontiers Group today named Jennifer Doudna, Ph.D., at the University of California, Berkeley an Allen Distinguished Investigator (ADI) with an award for “Antiviral Machinery and Cell Editing Platforms” at the frontier of RNA targeting and cell editing. Her work will build on powerful technology to edit the genome of any organism and seek to discover improved ways to manipulate DNA and novel ways to target RNA in cells, with significant impact on the treatment of disease and management of the environment. The grant is funded at \$1.5 million over three years.

“We are thrilled to see Jennifer turning her pioneering vision on a distant new frontier—the idea that targeting RNA might offer an even more powerful way than direct gene editing to program cell behaviors and manage disease,” says Tom Skalak, Ph.D., Executive Director of The Paul G. Allen Frontiers Group. “Shifting this fundamental boundary of biological knowledge could be game-changing, and bring benefits to millions of people.”

Doudna was one of the early discoverers of CRISPR-Cas9: recurring palindromic gene sequences in bacteria that, in partnership with a protein called Cas9, help bacteria defend themselves against viral infection. This fundamental science research led to what is now one of the most powerful ways to add or remove genes to virtually any genome, far more easily and less expensively than previous methods. The CRISPR-Cas9 technology was heralded as the 2015 Breakthrough of the Year by *Science* magazine.

“Having the opportunity to open new directions and expand our knowledge of what CRISPR technology can do is incredibly exciting,” says Doudna, Professor of Chemistry and of Molecular and Cell Biology at the University of California, Berkeley. “By taking advantage of our unique resources and tapping into the knowledge of how organisms defend themselves against bacterial infection, we hope to learn new fundamental biology and create additional cell editing tools that consider the whole system of both RNA and DNA function.”

Editing gene sequences like those in human genomes is still challenging in part because some genetic material is tightly wound within chromatin, making it difficult for scientists to reach many genes of interest. To overcome this, Doudna’s research will look beyond bacterial Cas9, which is used in most methods, to similar proteins in different organisms.

Other proteins completely separate from Cas9 may also play similar roles but with potential benefits in biotechnology applications. Even more radical, targeting RNA instead of genomic DNA may allow for cell editing manipulations not previously possible.

“Jennifer Doudna’s pioneering new vision for RNA targeting and novel DNA editing strategies has the potential to change the world,” says Nicholas B. Dirks, Ph.D., Chancellor of the University of California, Berkeley. “Her

way of working is a role model for pioneers everywhere because it shows how exploring basic science can produce surprising and disruptive technology that improves lives around the world.”

The work could also revolutionize how we study living cells. Because Cas9 and analogous proteins bind to strands of RNA, tagging the protein with a fluorescent marker could enable scientists to observe RNA in cells in real time—an important step in unraveling the complexity of many cellular behaviors. Targeting RNA would offer a way to edit cell behaviors without targeting the genome directly, opening up a vast new frontier.

The Paul G. Allen Frontiers Group seeks to open new frontiers in science, and the ADI program supports early-stage research with the potential to reinvent entire fields. By using curiosity-driven foundational research and insights to expand the reach of gene editing and open RNA targeting, Doudna’s work has the potential to make dramatic advances in cell editing technologies to fight disease, improve agriculture and promote environmental health.

About The Paul G. Allen Frontiers Group

The Paul G. Allen Frontiers Group is dedicated to exploring the landscape of science to identify and fund pioneers with ideas that will advance knowledge and make the world better. Through continuous dialogue with scientists across the world, The Paul G. Allen Frontiers Group seeks opportunities to expand the boundaries of knowledge and solve important problems. Programs include the Allen Discovery Centers at partner institutions for leadership-driven, compass-guided research, and the Allen Distinguished Investigators for frontier explorations with exceptional creativity and potential impact. The Paul G. Allen Frontiers Group was founded in 2016 by philanthropist and visionary Paul G. Allen. For more information visit allenfrontiersgroup.org.

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