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Geneticists Emmanuelle Charpentier and Jennifer Doudna Share \$500,000 Gruber Genetics Prize for Landmark Discovery of the RNA-guided CRISPR/Cas9 Gene Editing System



Emmanuelle Charpentier



Jennifer Doudna

June 16, 2015, New Haven, CT – The 2015 Gruber Genetics Prize will be awarded this year to microbiologist Emmanuelle Charpentier, PhD, of the Helmholtz Centre for Infection Research in Braunschweig, Germany and biochemist Jennifer Doudna, PhD, of the University of California, Berkeley. These two eminent scientists are being recognized for their joint creation of a revolutionary gene-editing technology known as CRISPR-Cas9, which functions as a molecular scissor, generating double-stranded cuts in targeted DNA molecules with exceptional precision. The technology is being used around the world to advance biological research and to engineer genes for developing powerful new therapies for a wide range of human diseases, as well as new biofuels and agricultural products.

The award will be presented to Charpentier and Doudna in Baltimore, Md., on Oct. 9 at the annual meeting of the American Society of Human Genetics.

“The discovery of the CRISPR-Cas9 cellular defense system has transformed molecular genetics,” said Utpal Banerjee, a member of the Selection Advisory Board to the Prize. “We now have a quick and highly accurate technology for deleting or adding specific pieces of DNA, an advance with wide-ranging implications for both basic science and clinical medicine.”

Charpentier and Doudna began their collaboration in 2011 after meeting at a scientific conference in Puerto Rico. Both had been trying to unlock the molecular mysteries of the CRISPR systems, an unusual repeating sequence of DNA that enables bacteria to mount a successful defense against viral invaders.

Charpentier was focusing on the type II system (CRISPR-Cas9) and had described in a seminal paper published in Nature in 2011, the three components of the system that consist of two RNAs forming a duplex (tracrRNA and crRNA) and the protein Cas9 (formerly named Csn1) and the roles of each component in the early steps of activation of the system. A year later, in their seminal 2012 paper in Science, Charpentier and Doudna showed that an RNA-guided protein, Cas9, “reads” genetic information on CRISPR sequences and then uses that information to seek out and destroy the invading viral DNA, essentially by cutting it up. The two scientists also demonstrated that this cellular defense system had applications beyond killing viruses, for the RNA could be engineered to attach to any gene, thus encouraging the Cas9 protein to cleave that spot. That made the CRISPR system an exceptionally powerful tool, for it could be customized to delete or add specific strands of DNA.

“Drs. Charpentier and Doudna have made an extraordinary contribution to molecular biology and genetics,” says Huda Zoghbi, chair of the Selection Advisory Board to the Prize. “Their research has led to an entire new approach to genome editing, which promises to open up new strategies for biological studies in many diverse organisms and for potentially treating diseases as varied as HIV/AIDs, sickle cell anemia, and muscular dystrophy. It’s a great honor to be awarding them with this prestigious award.”

Additional Information

In addition to the cash award, each recipient will receive a gold laureate pin and a citation that reads:

The Gruber Foundation proudly presents the 2015 Genetics Prize to Emmanuelle Charpentier and Jennifer Doudna for establishing a framework for universal genome editing.

Charpentier and Doudna discovered that the bacterial enzyme Cas9 is an endonuclease that cuts DNA at sites specified by a guide RNA, and defined biochemically the components required for this reaction. They showed that the sequence of the guide RNA could be modified to target the endonuclease to virtually any site. This provided the mechanism by which bacteria acquire immunity to specific viral infections, allowed introduction of specific mutations at desired sites, and provided the means to transfer efficient Cas9-directed break, repair, and editing to any cell type. This method has broadly enabled genome editing for uses in basic biology, medicine, biotechnology, and agriculture.

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Laureates of the Gruber Genetics Prize:

- **2014: Victor Ambros, David Baulcombe, and Gary Ruvkun**, for pioneering the study of small non-coding RNA’s, molecules that are recognized as playing a critical role in regulating gene expression
- **2013: Svante Pääbo**, for pioneering the analysis of ancient DNA
- **2012: Douglas C. Wallace**, for his groundbreaking contributions to mitochondrial genetics
- **2011: Ronald Davis**, for his pioneering development and application of recombinant-DNA techniques
- **2010: Gerald Fink**, whose work in yeast genetics advanced the field of molecular genetics

- **2009: Janet Davison Rowley**, for her seminal discoveries in molecular oncology
- **2008: Allan C. Spradling**, for his work on fly genomics
- **2007: Maynard V. Olson**, for his contributions to genome science
- **2006: Elizabeth H. Blackburn**, for her studies of telomeres and telomerase, and her science advocacy
- **2005: Robert H. Waterston**, for his pivotal role in the Human Genome Project
- **2004: Mary-Claire King**, for three major findings in modern genetics: the similarity of the human and chimpanzee genomes, finding a gene that predisposes to breast cancer, and forensic genetics.
- **2003: David Botstein**, a driving force in modern genetics who established the ground rules for human genetic mapping
- **2002: H. Robert Horvitz**, who defined genetic pathways responsible for programmed cell death
- **2001: Rudolf Jaenisch**, who created the first transgenic mouse to study human disease

The Prize recipients are chosen by the Genetics Selection Advisory Board. Its members are: **Bonnie Bassler**, Princeton University; **Utpal Banerjee**, University of California Los Angeles; **Martin Chalfie**, Columbia University; **Kay Davies**, University of Oxford; **Helen Hobbs**, University of Texas Southwestern; **Richard Lifton**, Yale School of Medicine; and **Huda Zoghbi**, Baylor College of Medicine (Chair).

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By agreement made in the spring of 2011 The Gruber Foundation has now been established at Yale University.

The Gruber International Prize Program honors individuals in the fields of Cosmology, Genetics and Neuroscience, whose groundbreaking work provides new models that inspire and enable fundamental shifts in knowledge and culture. The Selection Advisory Boards choose individuals whose contributions in their respective fields advance our knowledge and potentially have a profound impact on our lives.

The Genetics Prize is presented to a leading scientist, or up to three, in recognition of groundbreaking contributions to any realm of genetics research.

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For more information on the Gruber Prizes, visit www.gruber.yale.edu, e-mail info@gruber.yale.edu or contact A. Sarah Hreha at +1 (203) 432-6231. By mail: The Gruber Foundation, Yale University, Office of Development, PO Box 2038, New Haven, CT 06521.

Media materials and additional background information on the Gruber Prizes can be found at our online newsroom: www.gruber.yale.edu/news-media