

News Release

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Merck KGaA, Darmstadt, Germany, Awarded Australian CRISPR Nickase Patent for Foundational Genome-Editing Technology

- **Patent covers paired Cas9 nickase technology to reduce off-target effects, advance gene therapy and research**
- **Expands company's foundational CRISPR cutting and integration IP necessary to correct genetic defects in gene therapy patients**
- **Merck KGaA, Darmstadt, Germany to license CRISPR-related patents to interested parties**

Darmstadt, Germany, August 15, 2018 – [Merck](#) KGaA, Darmstadt, Germany, the vibrant science and technology company, today announced that the Australian Patent Office has allowed the company's patent application for the use of paired CRISPR nickases. Paired nickases represent a significant step in increasing safety by driving specificity through a highly flexible and efficient approach to reduce off-target effects. This improves CRISPR's ability to fix diseased genes while not affecting healthy ones.

"We've made tremendous strides in recent years evolving CRISPR technology, and this is a pivotal time in scientific research," said Udit Batra, member of the Executive Board and CEO, Life Science business of Merck KGaA, Darmstadt, Germany. "Our paired nickase CRISPR technology is important for researchers who need highly accurate methods when developing treatments for difficult-to-treat diseases. This new patent allowance represents a significant advancement in safety for CRISPR-enabled therapeutics."

Page 1 of 3



The allowed patent application covers a foundational CRISPR strategy in which two CRISPR nickases are targeted to a common gene target and work together by nicking or cleaving opposite strands of a chromosomal sequence to create a double-stranded break. This process can optionally include an exogenous or donor sequence for insertion in the same manner as the company's patented CRISPR integration technology.

In addition to allowing a patent application on paired nickases, the Australian Patent Office recently announced the formal grant of the 2017 CRISPR integration patent filed by the Life Science business of Merck KGaA, Darmstadt, Germany, following the withdrawal of four independent, anonymously filed oppositions.

The CRISPR integration patent portfolio of the Life Science business of Merck KGaA, Darmstadt, Germany includes granted patents in Australia, Canada, China, Europe, Israel, Singapore and South Korea. These CRISPR patents are directed to chromosomal integration, or cutting of the sequence of eukaryotic cells and insertion of a synthetic exogenous DNA sequence to make a desired genomic change.

Paired CRISPR nickase methods build on other technologies in the patent portfolio of the Life Science business of Merck KGaA, Darmstadt, Germany, including CRISPR integration. Commercial organizations need this intellectual property for CRISPR-based insertion of DNA if they seek to correct genetic defects in the somatic cells of gene therapy patients. The Life Science business of Merck KGaA, Darmstadt, Germany is licensing this patent portfolio for all fields of use.

As the Life Science business of Merck KGaA, Darmstadt, Germany has been highly involved in genome-editing innovation for the past 14 years, the company recognizes that genome editing has resulted in major advancements in biological research and medicine. At the same time, the growing potential of genome-editing technologies has opened scientific, legal and societal concerns. As both a user and supplier of genome-editing technology, the businesses of Merck KGaA, Darmstadt, Germany support research with genome editing under careful consideration of ethical and legal standards. Merck KGaA, Darmstadt, Germany has established a [Bioethics Advisory Panel](#) to provide guidance for research in which its businesses are involved, including research on or using genome editing, and has defined a clear

operational [position](#) taking into account scientific and societal issues to inform promising therapeutic approaches for use in research and applications.

The Life Science business of Merck KGaA, Darmstadt, Germany was the first to offer custom biomolecules for genome editing globally (TargeTron™ RNA-guided group II introns and CompoZr™ zinc finger nucleases), driving adoption of these techniques by researchers all over the world. It was also the first to manufacture arrayed CRISPR libraries covering the entire human genome, accelerating disease cures by allowing scientists to explore more questions about root causes.

In addition to basic genome-editing research, the Life Science business of Merck KGaA, Darmstadt, Germany supports development of gene- and cell-based therapeutics and manufactures viral vectors. In 2016, the company launched a genome-editing initiative aimed at advancing research in novel modalities — from genome editing to gene medicine manufacturing — through a dedicated team and enhanced resources, further solidifying its commitment to the field.

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About Merck KGaA, Darmstadt, Germany

Merck KGaA, Darmstadt, Germany is a leading science and technology company in healthcare, life science and performance materials. Around 53,000 employees work to further develop technologies that improve and enhance life – from biopharmaceutical therapies to treat cancer or multiple sclerosis, cutting-edge systems for scientific research and production, to liquid crystals for smartphones and LCD televisions. In 2017, Merck KGaA, Darmstadt, Germany, generated sales of € 15.3 billion in 66 countries. Founded in 1668, Merck KGaA, Darmstadt, Germany is the world's oldest pharmaceutical and chemical company. The founding family remains the majority owner of the publicly listed corporate group. Merck KGaA, Darmstadt, Germany holds the global rights to the „Merck“ name and brand. The only exceptions are the United States and Canada, where the company operates as EMD Serono, MilliporeSigma and EMD Performance Materials.