

News Release

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Merck KGaA, Darmstadt, Germany, Grants Exclusive License to Vertex for Two DNA Damage Response Inhibitors

- **Exclusive license grants Vertex one clinical and one pre-clinical DNA-PK inhibitor for use in gene-editing applications in six specific genetic disease areas**
- **Merck KGaA, Darmstadt, Germany retains rights to both compounds in all other disease areas, including oncology**
- **Merck KGaA, Darmstadt, Germany retains the rights to utilize and license this technology in gene editing outside of the six specified disease areas**

Darmstadt, Germany, January 24, 2019 – Merck KGaA, Darmstadt, Germany, a leading science and technology company, which operates its healthcare business in the U.S. and Canada as EMD Serono, today announced that it has entered into an exclusive licensing agreement with Vertex Pharmaceuticals Incorporated, Boston, USA (NASDAQ: VRTX), for two DNA-dependent protein kinase (DNA-PK) inhibitors – M9831 (formerly known as VX-984) and an additional pre-clinical compound – in the field of gene editing for six specific genetic disease indications. Merck KGaA, Darmstadt, Germany will receive an upfront payment in addition to milestones and royalties on future net sales and retains the rights to both assets in all other disease areas including oncology, with the ability to develop both these compounds in-house, or to license them to future partners in the gene editing field. Vertex has the option to add indications to the license grant. Both molecules were acquired in a

Page 1 of 3



News Release

licensing agreement from Vertex in 2017, and are part of the company's broad portfolio of DNA Damage Response (DDR) inhibitors.

"This transaction illustrates our determination to maximize value creation from our pipeline," said Belén Garijo, Member of the Executive Board and CEO Healthcare, Merck KGaA, Darmstadt, Germany. "We are rapidly advancing our leading-edge DDR portfolio in oncology and are delighted to see the potential benefit of DNA-PK in genetic diseases through the enhancement of CRISPR/Cas9-mediated gene editing."

Merck KGaA, Darmstadt, Germany is investing significant resources into the promising area of DDR, and has considerable expertise and experience in developing DDR molecules, with the objective of becoming one of the leading players in this therapeutic area. The company is currently investigating four DDR molecules, including two ATR inhibitors, an ATM inhibitor and an investigational small-molecule of DNA-PK. DNA-PK is a key enzyme that could potentially enhance the efficacy of many commonly used DNA-damaging agents such as radiotherapy and chemotherapy.

Pre-clinical studies have shown that DNA-PK inhibitors can enhance CRISPR/Cas9-mediated gene editing. CRISPR/Cas9 is a technology used to modify genetic sequences and is being investigated for the treatment of various genetic disorders. This collaboration licenses two compounds to study the potential DNA-PK-inhibitor-mediated enhancement of gene editing for the treatment of six genetic diseases included in the license grant to Vertex.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious and life-threatening diseases. In addition to clinical development programs in CF, Vertex has more than a dozen ongoing research programs focused on the underlying mechanisms of other serious diseases.

Founded in 1989 in Cambridge, Mass., Vertex's headquarters is now located in Boston's Innovation District. Today, the company has research and development sites and commercial offices in the United States, Europe, Canada, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including being named to Science magazine's Top Employers in the life sciences ranking for nine years in a row.

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News Release

About Merck KGaA, Darmstadt, Germany

Merck KGaA, Darmstadt, Germany, a vibrant science and technology company, operates across healthcare, life science and performance materials. Around 51,000 employees work to make a positive difference to millions of people's lives every day by creating more joyful and sustainable ways to live. From advancing gene editing technologies and discovering unique ways to treat the most challenging diseases to enabling the intelligence of devices – the company is everywhere. In 2017, Merck KGaA, Darmstadt, Germany, generated sales of € 15.3 billion in 66 countries.

The company holds the global rights to the name and trademark "Merck" internationally. The only exceptions are the United States and Canada, where the business sectors of Merck KGaA, Darmstadt, Germany operate as EMD Serono in healthcare, MilliporeSigma in life science, and EMD Performance Materials. Since its founding in 1668, scientific exploration and responsible entrepreneurship have been key to the company's technological and scientific advances. To this day, the founding family remains the majority owner of the publicly listed company. For more information about Merck, KGaA, Darmstadt, Germany, visit www.emdgroup.com.