

## Merck partners Vertex for two DNA damage response inhibitors

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**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, a leading science and technology company, has entered into an exclusive licensing agreement with Vertex Pharmaceuticals 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 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 licensing agreement from Vertex in 2017, and are part of Merck's broad portfolio of DNA Damage Response (DDR) inhibitors.

"This transaction illustrates our determination to maximize value creation from our pipeline," said Belen Garijo, Member of the Executive Board and CEO Healthcare, Merck. "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 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.