



## **Agora Open Science Trust Announces Nomination of M4K2009 as Lead Development Candidate for Pediatric Brain Cancer Therapy**

*A major milestone in open science drug discovery is marked by the advancement of M4K Pharma's ALK2 inhibitor program into IND-enabling studies for Diffuse Intrinsic Pontine Glioma (DIPG).*

*The advancement was supported by Conscience's Developing Medicine through Open Science (DMOS) program, which enables collaborative, early-stage research in the hopes of accelerating development of treatments for rare diseases.*

**Toronto, ON, Canada – December 8, 2025** - Agora Open Science Trust today announced the nomination of M4K2009 as the lead development candidate for its M4K Pharma program, which applies open science to drive the development of an affordable treatment for Diffuse Intrinsic Pontine Glioma (DIPG), a rare and devastating pediatric brain cancer. Nominating a lead development candidate marks the point where a discovery program selects a single molecule with the highest potential to advance further towards human clinical trials. The selection of M4K2009 represents a significant scientific and organizational milestone.

The nomination follows an extensive, multi-year research collaboration with several academic and industry partners worldwide. Among the hundreds of compounds designed and evaluated, M4K2009 demonstrated excellent potency, selectivity, brain penetration, and tolerability in preclinical models, establishing it as a strong candidate for further development.

"Our nomination of M4K2009 represents an important milestone for open science drug discovery," said Max Morgan, CEO of Agora Open Science Trust. "By pooling resources, foregoing patents and secrecy, and instead working openly and collaboratively across institutions with complementary capabilities and expertise, our team has now shown that open science is capable of delivering high-quality, clinically viable candidates,

particularly in areas of market failure underserved by traditional proprietary approaches.”

With financial and in-kind support from the Krembil Foundation, Conscience’s Developing Medicines through Open Science (DMOS) program, OICR’s Cancer Therapeutics Innovation Pipeline (CTIP), Charles River Laboratories, Structural Genomics Consortium, Institute of Cancer Research, University of Pennsylvania, Montreal Children’s Hospital/McGill University, CAMH, Children’s Cancer Institute, Sant Joan de Deu Hospital, Reaction Biology, GL Chemtec and The Brain Tumour Charity, M4K Pharma has made critical strides toward realizing an affordable, first-in-class therapy for children affected by DIPG.

Conscience’s DMOS program, which is supported by a grant from the Government of Canada’s Strategic Innovation Fund, enables collaborative preclinical drug discovery in areas where open sharing and collaboration are key to advancement towards accessible treatments, such as rare and neglected diseases. Participating projects which include at least one Canadian SME, use an open science approach as they foster collaboration for pre-clinical work, demonstrate early proof-of-concept research, and ultimately translate innovation into affordable medicine and better health outcomes globally. The inaugural round of the program launched in February 2025, with the Agora Open Science Trust as one of the recipients.

“M4K Pharma’s lead development candidate nomination represents a significant step in advancing the program toward the clinical stage and demonstrates that an open science approach can bring us closer to meaningful treatments for rare diseases like DIPG,” says Anne Fortier, VP drug discovery and development at Conscience. “Through the DMOS program, we’re proud to support rigorous, collaborative work that helps make therapies more accessible and affordable, particularly in areas where few solutions currently exist.”

“Canada is proud to support open science collaborations that deliver real hope for children and families facing rare diseases like DIPG. The nomination of M4K2009 as a lead candidate reflects the power of Canadian innovation and partnership. By accelerating the development of affordable and accessible therapies, we are not only advancing research, but strengthening our position as a global leader in health innovation for the benefit of all Canadians,” said The Honourable Mélanie Joly, Minister of Industry and Minister responsible for Canada Economic Development for Quebec Regions.

By sharing data and results with the research community and firmly committing to Agora’s charitable mission of achieving affordable access for affected children, the M4K

Pharma program has been successful in motivating and aligning a broad range of contributors. The program has crowdsourced inputs from leading experts and reduced costs and duplication of effort across ALK2 drug discovery research for DIPG, and demonstrated that open science can achieve breakthroughs in areas that traditional, closed models often overlook.

For more information, visit <https://m4kpharma.com> or <https://agoraopensciencetrust.org>.

### **About Agora Open Science Trust**

Agora Open Science Trust is a Canadian charity whose mission is to accelerate the discovery and development of affordable new medicines through open science. Agora's first initiative – M4K Pharma ('Medicines for Kids') – is using open science to drive preclinical and clinical development of a novel ALK2 inhibitor for the treatment of Diffuse Intrinsic Pontine Glioma (DIPG), a rare pediatric brain cancer. Agora's pipeline of collaborative open science drug discovery programs has recently expanded to include programs for Spinal Bulbar Muscular Atrophy (SBMA), a rare genetic neuromuscular disorder, and Primary Sclerosing Cholangitis (PSC), a rare liver disease – both of which currently have no approved treatment.

Agora continues to welcome collaborative funding partners whose support will help advance M4K2009 into clinical evaluation and sustain its mission to develop affordable medicines through open science. You can support its mission at <https://www.agoraopensciencetrust.org/donate-to-our-mission>.

### **About M4K Pharma**

M4K Pharma is an open science drug development company wholly owned by Agora Open Science Trust. Agora incorporated M4K Pharma to pursue commercial development of the ALK2 clinical candidate arising from its collaborative DIPG research program. M4K Pharma is tasked with leveraging regulatory exclusivity rights and priority review voucher assets to attract late-stage commercial investment and partnerships focused on achieving affordable access to a clinically validated ALK2 inhibitor. Any M4K Pharma profits from these commercial efforts will be distributed to the Agora charity to support DIPG patient access initiatives and further open science research programs.

### **About Conscience**

Conscience is a non-profit focused on enabling drug discovery and development in areas where open sharing and collaboration are key to advancement towards accessible treatments. It does so by encouraging and funding the open sharing of

knowledge and tools, the use and improvement of artificial intelligence, and the development of policies that break down barriers of traditional drug development. Powered by a network that includes academics, industry, technologists, policy experts, and public support, Conscience seeks to drive innovation by turning drug discovery and development into a team sport. Its open science model brings unique value in areas where market solutions are limited, offering alternatives to traditional intellectual property models to make new accessible medicines so no one is left behind. Through key initiatives, such as its DMOS (Developing Medicines through Open Science) program and CACHE (Critical Assessment of Computation Hit-finding Experiments) Challenges, Conscience is accelerating the path to treatments for those who need them most. For more information, visit [conscience.ca](https://conscience.ca).

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