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October 17, 2018

RE: Standing Committee on Health Study M-132
Structural Genomics Consortium Briefing Note: Specific Policy Recommendations

Dear Members of the Standing Committee on Health,

In our appearance before the Standing Committee on October 16, 2018, we spoke about how the Structural Genomics Consortium (SGC) (https://www.thesgc.org/) has been highly successful in using open science to accelerate downstream drug discovery. So successful in fact that it has the backing of many of the world's largest pharmaceutical companies, who have invested over $200 million dollars into the SGC since its inception. And the more open science projects we have undertaken, the more companies have decided to support us.

But we need to do more to create *affordable* new medicines. The dominant model of translating publicly funded science into commercial drug products in Canada, and elsewhere, relies heavily on patents, secrecy, and private investment to support drug discovery. But this model makes it very difficult to collaborate broadly and instead creates research silos that keep others in the dark about successes and failures, often for many years. This in turn leads to enormous redundancy, which not only multiplies costs many times over but also exposes far too many clinical trial participants to needless risk. At the same time, the model ignores many of the most promising avenues for discovery and development as either too risky or not sufficiently lucrative. The end product has been ever-fewer drugs approved per dollar of research investment and increasingly unsustainable prices for drugs that do reach the market.

In short, something clearly needs to change. So we described for the Standing Committee how open science can deliver the goods in drug discovery too, by removing impediments to knowledge flow and collaboration, preventing costly redundancy, bringing together divergent expertise and resources, motivating participation and funding for the public good, and improving the quality of science through transparency.

To lead the way here in Canada, in late 2017 we founded M4K Pharma Inc. (M4K) (https://m4kpharma.com/), an acronym for Medicines for Kids, the world's first open science drug discovery company. We told the Standing Committee about how M4K has attracted millions of dollars in funding and in-kind contributions from a broad range of collaborators in industry, academia, and philanthropy so far. We also told the Standing Committee about how M4K has already identified a promising lead drug candidate for a deadly childhood brain cancer called diffuse intrinsic pontine glioma (DIPG), thanks to these contributions.

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But we are not asking the Standing Committee to simply take our word for it. Even though we are leading in Canada, the promise of open science to efficiently deliver affordable health innovations is being increasingly recognized by a broad range of other stakeholders around the world. The National Institutes of Health (NIH) in the U.S. recently launched a large funding program for open Alzheimer's Disease drug discovery consortia.[1] The UCL Institute for Innovation and Public Purpose in the U.K. just released a detailed report calling for new business models to translate publicly-funded health research that are predicated on knowledge sharing and a commitment to affordable pricing.[2] The Bill and Melinda Gates Foundation hosted the Open Science Leadership Forum in October 2017 in Washington DC, which brought together delegates from the SGC, governments, science agencies, funding bodies, philanthropies, patient organizations, and the pharmaceutical and biotech industries to discuss how open science can advance research and innovation, including by leading to new product development.[3] Finally, in 2016 the Montreal Neurological Institute (MNI), one of the world's preeminent brain research centers, has entirely committed itself to open science to accelerate the discovery of novel therapies to treat patients suffering from neurological disease.[4]

Policy Recommendations

We provide this written submission in follow-up to our testimony to offer specific policy recommendations to the Standing Committee. In brief, what we are saying is that Canada should tweak its funding and regulatory environments to provide opportunities and incentives for companies like M4K Pharma to pursue open science drug development here in Canada. This would help keep Canada at the very forefront of this new model, which could offer dramatic benefits for human health.

Here are four proposals for how to do so:

First, Canada should ensure that public programs that support translation of biomedical discoveries into new medicines do not focus exclusively on proprietary, intellectual property-driven business models. Instead, space should be created for alternative open science models to be competitive for translational funding opportunities in Canada.

Second, Health Canada should offer an 'open drug development' designation as an inducement for companies to pursue an open science model and commit to affordable drug pricing.

This designation would operate similarly to orphan drug status for rare diseases in the US and EU, in that it would make open drug companies eligible for commercially valuable development incentives like clinical trial design assistance from Health Canada, Health Canada application fee waivers, tax credits for development costs, fast-track review of openly-developed products by Health Canada, and public grants to support open clinical trials.

Orphan drug designations in the US and EU have led to dramatic increases in rare disease drug development and product registrations in those markets,[5] but the resulting prices are exorbitant. A Canadian 'open drug development' designation could likewise spur the creation of new drugs but could do so in a manner that ensures affordability.

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[1] https://grants.nih.gov/grants/guide/notice-files/NOT-AG-18-025.html

[2] UCL Institute for Innovation and Public Purpose (2018), 'The people's prescription: Re-imagining health innovation to deliver public value', *IIPP Policy Report,* 2018-10. London: IIPP, Global Justice Now, Just Treatment, STOPAIDS. Available at: https://www.ucl.ac.uk/bartlett/public-purpose/wp2018-10

[3] Ali-Khan SE, Jean A, MacDonald E and Gold ER. Defining Success in Open Science [version 2; referees: 2 approved]. MNI Open Res 2018, 2:2 (doi: 10.12688/mniopenres.12780.2).

[4] https://www.mcgill.ca/neuro/open-science-0/about-open-science

[5] Canadian Agency for Drugs and Technology in Health (2016), 'Drugs for Rare Diseases: Evolving Trends in Regulatory and Health Technology Assessment Perspectives', Ottawa, CADTH (Environmental scan; issue 42). Available at: https://www.cadth.ca/drugs-rare-diseases-evolving-trends-regulatory-and-health-technology-assessment-perspectives

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Third, Canada should invest in enabling digital infrastructure for open drug discovery.

Specifically, we need an open data repository to help companies who want to publicly share their preclinical and clinical data during the drug development process – i.e. before their final regulatory applications are submitted to Health Canada. As the EU has already done, Health Canada is currently implementing a mandatory system for public release of clinical trial data after regulatory consideration. This is a commendable initiative but will still delay the benefits of open data for years in most cases.

Open science companies like M4K Pharma need infrastructure for voluntary data sharing much earlier, but nothing currently exists. Canada could lead the way by creating it. And Health Canada could encourage companies to share their data in this new repository during development by offering a period of protection for deposited datasets. During this period, competitors would be precluded from using the data in a regulatory submission designed to leapfrog the depositor's own marketing application.

Finally, and we believe most importantly, Health Canada should offer an 'open science' extension of innovative drug status by amending section C.08.004.1 of the *Food and Drug Regulations*.

As we described in our testimony, drugs with new active ingredients - which are designated as "innovative drugs" by Health Canada - are eligible for 8 years of protection against generic competition in Canada (soon to be 10 years for a class of complex therapeutics called biologics, thanks to the USMCA). This period of exclusivity on the market is a strong incentive for drug development that is completely separate from patent protection. And, unlike patents, it is fully consistent with open science, which makes it a particularly crucial incentive for open drug developers like M4K.

In fact, Health Canada already offers a 6-month extension of innovative drug status to create incentives for pediatric studies through section C.08.004.1(3) of the *Food and Drug Regulations*. We should create another form of extension for open science drug development, one that is significantly longer given that companies utilizing open science will not have concurrent patent protection to stave off competition. This exclusivity extension should only be available to a company that can meet three criteria:

1. It can establish that it openly deposited its data into the Health Canada repository we recommend creating above, or into a comparable open database.

2. The company certifies that it has not sought patents on its product, rendering it ineligible to list patents on Health Canada's patent register (which is an important tool for pharmaceutical companies to enforce their patents).

3. The company enters into an agreement with Health Canada on an affordable price for its drug in Canada, with which the company would need to adhere to maintain its exclusivity. A body like the Canadian Agency for Drugs and Technology in Health (CADTH) could be enlisted to help determine appropriate price levels to be negotiated in these agreements.

The four policy recommendations we offer here have the significant benefit of being voluntary programs that would leave the current proprietary, patent-based system entirely intact. At the same time, these initiatives could help steer some resources in the system towards a more efficient and more cost-effective way to create affordable medicines: open science. We suggest that Canada could experiment by initially targeting these funding and incentive mechanisms towards disease areas that are currently ignored or underserved by industrial efforts, for example: neurodegeneration, infectious diseases, tropical diseases, pediatric diseases, and rare genetic disorders. Once data accumulate to support the open model, these initiatives could be expanded more broadly.

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We thank the Standing Committee for considering our recommendations and would invite any follow up questions that Committee Members may have.

Yours sincerely,

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