Could Open be the Yellow Brick Road to Precision Medicine?

An Overview of Open Models of Collaboration in Genomics and Precision Medicine

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In Canada, one in five adults suffer from a chronic disease. Major chronic diseases (cardiovascular diseases, cancer, chronic respiratory diseases, and diabetes) cause 65% of the country's deaths. Healthcare expenditures for these chronic diseases, along with mood and anxiety disorders, account for one third of the country's direct healthcare expenditures.[1] According to the U.S. Centers for Disease Control and Prevention (CDC), in 2012, about half of the U.S, population suffered from one or more chronic health conditions and one in every four adults had two or more chronic health conditions. Seven of the top ten causes of death in 2014 were chronic diseases. Heart disease and cancer alone were responsible for 46% of all deaths. 86% of the total healthcare expenditure in the U.S. is devoted to treat people with chronic and mental health conditions.[2] In Mexico, chronic diseases are also one of the main causes of death in adults. Likewise, the treatments for these conditions are very burdensome on the public healthcare system and on the population using the private healthcare system.[3] The healthcare agencies in the three countries agree that the mortality and morbidity numbers and the costs associated with their treatments could be reduced in the long-term if preventative measures were adopted.

Precision medicine could improve prevention and promote better healthcare.[4] A number of genetic tests already exist that could help with an early diagnosis, a most likely prognosis, and the development of the necessary and better-suited treatments. However, outside of niche markets such as rare diseases (including some cancers) and pharmacogenomics, the ultimate potential of OMICS sciences in the development of precision medicine remains uncertain. Furthermore, the development of precision medicine brings about economic challenges, such as costly development, high rate of failure, and reduced market size (in comparison to other drug development). These challenges explain, in part, the outrageous price of some of the new biological drugs.[5]

In order to advance precision medicine, a growing number of projects are adopting models that are more open (i.e. open science, open source, peer-based production, protected commons, crowdsourcing, open innovation, etc.) than the "traditional" drug development one based on proprietary intellectual property. Some of these projects are

[1] Public Health Agency of Canada, "How Healthy are Canadians?", (8 March 2017), online: *Gov Can* <https://www.canada.ca/en/public-health/services/publications/healthy-living/how-healthy-canadians.html>.

[2] Centers for Disease Control and Prevention, "Chronic Disease Overview. Chronic Disease Prevention and Health Promotion", (28 June 2017), online: <https://www.cdc.gov/chronicdisease/overview/index.htm>.

[3] Subsecretaría de Integración y Desarrollo del Sector Salud Dirección General de Evaluación del Desempeño Secretaria de Salud, *Informe Sobre la Salud de los Mexicanos 2015. Diagnostico General de la Salud Poblacional.* (Secretaria de Salud, 2015) at 78–80; Fanny Miranda, "Gasta el IMSS 80 mil mdp en 4 enfermedades crónicas", *Milenio* (8 September 2016), online: <http://www.milenio.com/cultura/Gasta-IMSS-mil-enfermedades-cronicas_0_807519254.html>.

[4] A D Stern, B M Alexander & A Chandra, "How economics can shape precision medicines" (2017) 355:6330 Science 1131 at 1131.

[5] *Ibid* at 1131–1133.

Hortonworks Inc.'s consortium,[6] PrecisionFDA,[7] Arvados Project,[8] InnoCentive,[9] Structural Genomics Consortium,[10] and Montreal Neurological Institute and Hospital.[11]

Other factors have also encouraged companies and researchers to opt for more open models of collaboration. For instance, the capacity of the patent system to promote innovation in medicine and medical genetics has been increasingly questioned by experts.[12] Additionally, the separation between basic research (academia) and the development of clinical applications (industry) is becoming increasingly blurry, turning medical innovation into a more holistic process, where open models are used more downstream in the innovation process than ever before.[13] Moreover, a growing number of funding organizations are actively advocating for open approaches to research and innovation.[14]

This workshop provides the forum to discuss experiences, objectives, challenges, concerns, benefits, and potential paths to follow regarding the use of open models of collaboration in the field of personalized medicine in North America: two developed countries and one developing country.

[6] The Hortonworks consortium defines and develops an open source genomics platform to accelerate genomics-based precision medicine in research and clinical care. Michelle Lazzar, "Hortonworks Initiates Precision Medicine Consortium to Explore Next Generation Genomics Open Source Platform", (28 June 2016), online: *Hortonworks* <https://hortonworks.com/press-releases/hortonworks-initiates-precision-medicine-consortium-explore-next-generation-genomics-open-source-platform/>.

[7] PrecisionFDA is a community cloud-based platform for next generation assay evaluation where researchers, test developers, and community members can share and validate their tests and results against crowdsourced reference material. PrecisionFDA, "About precisionFDA", online: <https://precision.fda.gov/about>; Taha Kass-Hout & David Litwack, "Advancing precision medicine by enabling a collaborative informatics community", (5 August 2015), online: <https://blogs.fda.gov/fdavoice/index.php/2015/08/advancing-precision-medicine-by-enabling-a-collaborative-informatics-community/>.

[8] The Arvados core is an open source platform for the production and distribution of open source computing software for bioinformatics, data science, and very large datasets. "Arvados | Open Source Big Data Processing and Bioinformatics", (2016), online: *Arvados* <https://arvados.org/>.

[9] Innocentive is a platform that enables companies and researchers to obtain ideas and inputs from a broader network in a crowdsourcing model. "InnoCentive", (2017), online: *InnoCentive* <https://www.innocentive.com/>; Lilla Landeck et al, "The role of open innovation in biomarker discovery" (2016) 1:2 Adv Precis Med, online: <http://ojs.whioce.com/index.php/apm/article/view/89>.

[10] The Structural Genomics Consortium is a public-private partnership that supports the discovery of new medicines through open access genomics research. "SGC | Mission and Philosophy", online: *SGC* <http://www.thesgc.org/about/what_is_the_sgc>.

[11] The Montreal Neurological Institute and Hospital is becoming the first open science institute in the world to accelerate the discovery of therapies for patients suffering from neurological diseases. This endeavor includes providing open access to its C-BIG Repository of brain imaging, clinical, demographic, genetic, and cellular data, and samples, drug-discovery platform, and neuroinformatics platforms. "Open Science | Montreal Neurological Institute and Hospital - McGill University", online: *Montr Neurol Inst Hosp - McGill Univ* <https://www.mcgill.ca/neuro/open-science-0>.

[12] E Richard Gold et al, "Are Patents Impeding Medical Care and Innovation?" (2010) 7:1 PLOS Med 1; Arti K Rai et al, "Pathways across the valley of death: novel intellectual property strategies for accelerated drug discovery" (2008) 8:1 Yale J Health Policy Law Ethics 1; Amy Kapczynski, "Addressing Global Health Inequalities: An Open Licensing Approach for University Innovations" (2005) 20:2 Berkeley Technol Law J 1032; Aled Edwards, "To spark medical innovation, Canada should embrace Open Science", (7 January 2017), online: <https://www.theglobeandmail.com/report-on-business/rob-commentary/to-spark-medical-innovation-canada-should-embrace-open-science/article33533010/>; Lawrence Horn, "Patent pools for CRISPR technology" (2017) 355:6331 Science 1274; Subhashini Chandrasekharan & Robert Cook-Deegan, "Gene patents and personalized medicine - what lies ahead?" (2009) 1 Genome Med 92.

[13] Teri Melese et al, "Open innovation networks between academia and industry: an imperative for breakthrough therapies" (2009) 15:5 Nat Med 502; Martin Wehling, "Translational medicine: science or wishful thinking?" (2008) 6 J Transl Med 31; Jackie Hunter & Susie Stephens, "Is open innovation the way forward for big pharma?" (2010) 9:2 Nat Rev Drug Discov 87.

[14] Examples include: the Organisation for Economic Cooperation and Development (OECD) Principles and Guidelines for Access to Research Data from Public Funding, Organisation of the Human Genome (HUGO) Statement on Human Genomic Databases, Genome Canada, and the US National Institutes of Health Final Statement on Sharing Research Data.

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Centre de génomique et politiques

Program

8.00-9.00: Breakfast and opening

9.00-9.50: **Jerome Reichman** (Why the Nagoya Protocol to the CBD Matters to Medical Science and Industry in Canada? | 30 min presentation/20 min Q&A)

9.50-10.40: **Aled Edwards** (Open science: A better bargain between science and society| 30 min presentation/20 min Q&A)

10.40-11.00: Coffee Break

11.00-11.50: **Robert Cook Deegan** (Data-hoarding in genomics can kill. What can we do about it? | 30 min presentation/20 min Q&A)

11.50-12.40: **Megan Doerr** (mPower: A case study in open models of precision medicine | 30 min presentation/20 min Q&A)

12.40-13.40: Lunch

13.40-14.40: **Palmira Granados** (Finding a path through innovation's labyrinth of solitude. The case of 3D bioprinting | 30 min presentation/20 min Q&A)

14.40-16.00: **Round table** chaired by Yann Joly addressing next steps for Canada and the possibilities of reaching a synergistic statement

16.00: **Final remarks**

BIOGRAPHIES

Prof. Yann Joly, Workshop Chair

Yann Joly is a Lawyer Emeritus from the Quebec Bar and holds the position of Research Director at the Centre of Genomics and Policies (CGP). He is an Associate Professor at the Faculty of Medicine, Department of Human Genetics and at the Bioethics Unit, at McGill University. He is a research fellow from the Fonds de recherche du Québec- Santé (FRQS) and an associate researcher at the Centre de recherche en droit public (Université de Montréal). He also works as an ethics and legal consultant in the private sector. Prof. Joly is the Data Access Officer of the International Cancer Genome Consortium (ICGC). His research activities lie at the interface of the fields of intellectual property, health law (biotechnology and other emerging health technologies) and bioethics. He has served as a legal advisor on several ethics committees in the public and private sectors.

Prof. Joly is a member of the Scientific Committee of the legal journal Lex Electronica and an Advisory Board member of the Current Pharmacogenomics and Personalized Medicine Journal. He recently received the Quebec Bar Award of Merit (Innovation) for his work on the right to privacy in the biomedical field.

Prof. Jerome Reichman

Jerome H. Reichman is Bunyan S. Womble Professor of Law at Duke Law School. He has written and lectured widely on diverse aspects of intellectual property law, including comparative and international intellectual property law and the connections between intellectual property and international trade law. His articles in this area have particularly addressed the problems that developing countries face in implementing the World Trade Organization's Agreement on Trade-Related Aspects of Intellectual Property Rights (TRIPS Agreement). On this and related themes, he and Keith Maskus have recently published a book entitled *International Public Goods and Transfer of Technology Under a Globalized Intellectual Property Regime.*

Other recent writings have focused on intellectual property rights in data; the appropriate contractual regime for online delivery of computer programs and other information goods; and on the use of liability rules to stimulate investment in innovation. His most recent publication is Why the Nagoya Protocol to the CBD Matters to Science and Industry in Canada and the United States?

Professor Reichman serves as special advisor to the United States National Academies and the International Council for Science (ICSU) on the subject of legal protection for databases. He is a consultant to numerous intergovernmental and nongovernmental organizations; a member of the Board of Editors, *Journal of International Economic Law*; and on the Scientific Advisory Board of *Il Diritto di Autore* (Rome).

Prof. Aled Edwards

Aled Edwards is the founding and current CEO of the Structural Genomics Consortium (SGC), a charity that generates research tools to support basic science and drug discovery.

The SGC has been sharing its science freely since 2003 and is widely acknowledged as a pioneer in open science. In 2007 became the first research organization to adopt a policy not to file patents on any of its own research, or its collaborative research. Recently, the SGC created an "Extreme Open Science Unit" which comprises 20 early-stage researchers who are sharing their experimental data in real time on the internet. Last month, Aled founded M4K Pharma, an open source drug discovery company focused on childhood brain tumours.

Aled is a Professor at Toronto, Oxford and McGill Universities. He studied biochemistry at McGill and did his post-doctoral work at Stanford with Roger Kornberg. In 2015, he was elected a Senior Ashoka Fellow for social entrepreneurship.

Prof. Robert Cook-Deegan

Robert Cook-Deegan is a professor in the School for the Future of Innovation in Society, and with the Consortium for Science, Policy & Outcomes at Arizona State University. He founded and directed Duke's Center for Genome Ethics, Law & Policy 2002- 2012, and taught in Duke's in-Washington program through June 2016.

Before Duke he worked at the National Academies of Science, Engineering and Medicine 1991-2002; National Center for Human Genome Research (NIH) 1989-1990; and congressional Office of Technology Assessment 1982-1988. He obtained his MD from the University of Colorado in 1979; and a BA in chemistry (*magna cum laude*) from Harvard in 1975.

He is the author of *The Gene Wars: Science, Politics, and the Human Genome* and over 250 other publications.

Ms. Megan Doerr

A former botanist and middle school teacher, **Meg Doerr** joined the genetic counseling community in 2006. Meg led the clinical development and implementation of Cleveland Clinic's family history and risk assessment tool before joining the Governance team at Sage Bionetworks in 2015.

At Sage, Meg's efforts have been concentrated on supporting innovative, participant-centric approaches in open science. Her work has a strong focus on app-based research, including the ELSI issues associated with informed consent, research participation, and data sharing for secondary use in entirely remote, mobile platform-based research studies including for the *All of Us* Research Program.

Ms. Palmira Granados

Palmira Granados is a Mexican lawyer specialized in intellectual property and information technologies and a Doctor of Civil Law candidate at the Faculty of Law at McGill University under the supervision of Professor Richard Gold. Her interests focus on the intersection of intellectual property, ethics, human genetic information, health, and technology. She joined the Centre of Genomics and Policy (CGP) in 2013. Her work focuses on the social, ethical, and legal aspects of research and development involving human genetic information and technology.

Before joining the CGP, she became a member of the International Expert Group of the Innovation Partnership, of the New Researchers Group of VALGEN, and of the Centre for Intellectual Property and Policy of McGill University. Prior to Montreal, she obtained her law degree from la Escuela Libre de Derecho in Mexico and her LL.M from the Faculty of Law at the University of Toronto. She practiced law with a Mexican leading law firm in the area of intellectual property and information technology and was responsible for teaching the course Law and Public Policy at the at the Instituto Tecnologico Autonomo de Mexico. She has also been closely involved with the Free Software Foundation and Creative Commons Mexico.

Participants

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