Talk 1 by Feng Zhang:

Title: *Harnessing Microbial Diversity for Gene Editing and Beyond*

Abstract:
Precision genome editing, which can be used to alter specific DNA sequences, is a powerful tool for understanding the molecular circuitry underlying cellular processes. Over the past several years, we and others have harnessed microbial CRISPR-Cas systems for use as platforms for a range of genome manipulations, including single and multiplex gene knockout, gene activation, and large-scale screening applications. Recently, we discovered and characterized several novel CRISPR systems that target RNA, including the CRISPR-Cas13 family. We developed a toolbox for RNA modulation based on Cas13, including methods for highly specific RNA knockdown, transcript imaging, and precision base editing. During our initial characterization of Cas13, we observed that Cas13 also exhibits so-called non-specific "collateral" RNase activity in vitro, which we capitalized on to create SHERLOCK, a highly sensitive and specific CRISPR diagnostic platform. We are continuing to refine and extend CRISPR-based technologies as well as explore microbial diversity to find new enzymes and systems that can be adapted for use as molecular biology tools and novel therapeutics.

Bio:

Feng Zhang is a core institute member of the Broad Institute of MIT and Harvard, as well as an investigator at the McGovern Institute for Brain Research at MIT, the James and Patricia Poitras Professor of Neuroscience at MIT, and an associate professor at MIT, with joint appointments in the departments of Brain and Cognitive Sciences and Biological Engineering. Zhang is also an investigator at the Howard Hughes Medical Institute.

Zhang is a molecular biologist developing and applying novel molecular technologies for studying the brain. Zhang pioneered the development of genome editing tools for use in eukaryotic cells – including human cells – from natural microbial CRISPR-Cas9 systems. He and his team have adapted multiple other CRISPR systems for use as genome engineering tools, including most recently, the RNA-targeting CRISPR-Cas13 systems.

Zhang leverages CRISPR and other methods to study the genetics and epigenetics of human diseases, especially complex disorders, such as psychiatric and neurological diseases, that are caused by multiple genetic and environmental risk factors and which are difficult to model using conventional methods. His lab's tools, which he has made widely available, are also being used in the fields of immunology, clinical medicine and cancer biology, among others. His long-term goal is to develop novel therapeutic strategies for disease treatment.

Zhang is a recipient of many awards including the Canada Gairdner International Award, the Tang Prize, the Blavatnik National Award for Young Scientists, the Albany Medical Center Price in Medicine and Biomedical Research, and the Lemelson-MIT Prize. He has also received technology innovation awards from the Paul G. Allen Family, McKnight, New York Stem Cell, and Damon Runyon foundations. Zhang is an elected member of the National Academy of Sciences and the American Academy of Arts and Sciences.

Zhang received his A.B. in chemistry and physics from Harvard College and his Ph.D. in chemistry from Stanford University.

Further information is available at: https://www.broadinstitute.org/bios/feng-zhang

Talk 2 by Marie Louise (Marieke) De Bruin

Title: *Authorisation and regulatory decision-making about gene- and cell-based therapies*

Bio:

Marie Louise (Marieke) De Bruin is professor in Regulatory Science at the Faculty of Health and Medical Sciences of the University of Copenhagen, Denmark and director of the Copenhagen Centre for Regulatory Science (CORS) at the Department of Pharmacy.

Marie Louise (Marieke) De Bruin was trained as a pharmacist (Utrecht University) and epidemiologist (Erasmus University Rotterdam) and has combined academic research with working for regulatory authorities, both nationally (Pharmacovigilance expert for the Dutch Medicines Evaluation Board) and internationally (European Commission appointed independent Scientific Expert of the Pharmacovigilance Risk Assessment Committee (PRAC), at the European Medicines Agency 2012-2018). She has a strong background in epidemiology, is member of the steering group of the European Network of Centers for Pharmacoepidemiology and Pharmacovigilance (ENCePP) and has applied quantitative methods from epidemiology to study regulatory science research questions. Working as a regulator has given her theoretical training about and hands-on experience in the functioning of the regulatory system. She has wide experience in coordination and execution of public-private as well as academic research collaborations, nationally, at the EU level and globally.

Her personal motivation to engage in this field of research is to benefit public health and originates from her training as a health care professional. With her research, she wants to improve the drug regulatory system, by systematically studying its structure and behaviour as well as designing new tools to facilitate regulatory decision-making. Through improvement of

the drug regulatory system, she aims to contribute to an improvement of the health of the society.

Talk 3 by Esther van Zimmeren & Timo Minssen

Title: *Ethical Licensing for CRISPR: Exploring the Opportunities for Responsible Licensing Mechanisms to Restore Trust*

Abstract:
CRISPR has many promising applications. However, such promises also involve significant social responsibility for all actors involved. The recent controversy regarding the gene-edited twins has led to a public outcry and significant public concerns. Obviously, the actual risks and benefits of CRISPR will depend on the actual application (e.g. somatic gene editing v. germline gene editing). Yet, it is important to consider the different tools that are available to ensure that research and development in the field proceeds in a responsible way, including regulation, self-regulation and critical oversight. Ethical licensing of patented technologies can serve as one of the self-regulatory tools to limit potentially controversial use. This paper seeks to explore the opportunities for such ethical licensing for CRISPR. It starts off with a more general exploration of the concept of social responsibility and ethical licensing, followed by a short analysis of the CRISPR patent landscape and the current use of ethical licensing for CRISPR. We also cover the ongoing initiative of MPEG LA to set up a patent pool for CRISPR to deal with the increasingly complex and fragmented CRISPR patent landscape. Within the context of such a collaborative licensing mechanism, the impact of ethical licensing will be even more significant, but also more complex to manage from a governance perspective. Finally, the paper will also be framed within the literature on trust and new emerging technologies as ethical licensing could operate as an important signaling device for building/repairing trust in key pioneering technologies.

3. Bios of Esther & Timo

Esther van Zimmeren (LL.M. (2002), PhD (2011) KU Leuven) is a Research Professor in Intellectual Property (IP) Law & Governance at the University of Antwerp (UAntwerp, Research Groups Government & Law and Business & Law). She supervises an international and interdisciplinary team on IP Law & Governance consisting of 8 PhD researchers and 3 postdoctoral researchers. Esther teaches European, International and Comparative IP law and European internal market law and is a Coordinator of the Modules Law & International Business and Diversity & Law of the LL.M.-program at the UAntwerp. She is also a Visiting Professor at CeBIL. Esther has been the Head of the Research Group Government & Law (UAntwerp; 2015-2017) and a legal assistant at the General Court of the EU (2002-2004). She has particular expertise in IP law, in particular in patent law, IP governance, innovation

policy, technology transfer, competition law, contract law, European institutional and economic law and international trade law. Many of her research projects and publications are interdisciplinary; she has collaborations with e.g. geneticists, economists, physicists, philosophers, artists, designers, architects, urban planners, librarians and social and political scientists. She has three key interdisciplinary lines of research: Patenting & Licensing in the Biomedical Sector; Governance of the IP System and Open Metropolitan Design & Law.

Timo Minssen is Professor of Law at the University of Copenhagen (UCPH) and the Founder and Managing Director of UCPH's Center for Advanced Studies in Biomedical Innovation Law (CeBIL). His research concentrates on Intellectual Property-, Competition & Regulatory Law with a special focus on new technologies in the pharma, life science & biotech sectors. His studies comprise a plethora of legal issues emerging in the lifecycle of biotechnological and medical products and processes - from the regulation of research and incentives for innovation to technology transfer and commercialization. At UCPH he leads large interdisciplinary research projects examining legal issues in synthetic biology, precision medicine, biologics/biosimilars & large research infrastructures, as well as a large collaborative research program that is funded by the Novo Nordisk Foundation. He is scientific advisory board member of the Copenhagen Centre for Regulatory Sciences, steering committee member of the Danish Association for the Protection of Industrial Property and editorial board member of the European Pharmaceutical Law Review. Moreover, he acts as experts advisor in EU Commission studies. At CeBIL, Timo collaborates with an international network of renowned core-partners including inter alia the University of Cambridge, Harvard Law School and Harvard Medical School.

Panel discussion with the above speakers and Sven Bostyn, moderated by Janne Rothmar Herrmann

Bio of moderator and panelists:

Janne Rothmar Herrmann is professor with special responsibilities in health law and technology at the Faculty of Law, University of Copenhagen is member of the Center for

Advanced Studies in Biomedical Innovation Law. She conducts research ethics appraisals for the European Commission as an independent expert on H2020 projects, governor of the World Association of Medical Law, board member of the Danish Society for Medical Philosophy, Ethics and Methodology and alternate member of the Danish Committee of Scientific Misconduct by appointment by the Minister for Science.

Dr. Sven J.R. Bostyn (LLB, Lic. Jur., LLM law, PhD law) is Associate Professor of Biomedical Innovation Law at the Centre for Advanced Studies in Biomedical Innovation Law (CeBIL), Faculty of Law, University of Copenhagen. He also lectures at the Institute for Information Law (IVIR) of the University of Amsterdam, and is a regular guest professor at CEIPI in Strasbourg. He is specialized in all areas of IP law, with special emphasis on patent law relating to pharmaceuticals, biotechnology, medical devices, software, Artificial Intelligence and SPC's. Sven is one of the most prominent authorities in Europe in the area of patent law. Sven has also been in private practice for the last 14 years where he advised and advises clients on most aspects of patent law, competition law, licensing and regulatory exclusivities. He also acted and acts as an expert witness in court cases. He was member of a Scientific Advisory Committee at the Dutch Royal Academy of Sciences (Gene Patents Committee) and was from 2013-2016 the Chair of the Expert Committee at the European Commission (EC) on the development and implications of patent law in the field of biotechnology and genetic engineering, after having been a member of and Rapporteur at an earlier EC Expert Group (between 2003 and 2005). He has also advised both the Belgian and Dutch governments on policy regarding patentability of biotechnological and pharmaceutical inventions. He was recently one of the lead researchers in a study commissioned by the Dutch ministries of Health and Economic Affairs and Climate regarding protection mechanisms for pharmaceuticals.