BIOENGINEERING
Spring 2021 Seminar

Date: **Thursday, March 4, 2021**
Time: **12:00 pm - 1:00pm**
Location: **Virtual**
Join Zoom Meeting
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Meeting ID: 988 0549 4005 Passcode: 454698

Pieter Cullis, Ph.D.

Biography: Pieter R. Cullis, Ph.D. FRSC, FNAI (USA), Scientific Director & CEO, NanoMedicines Innovation Network, Canada's National Centre of Excellence in nanomedicines; Professor, Department of Biochemistry and Molecular Biology, University of British Columbia. Dr. Cullis and co-workers have been responsible for fundamental advances in the design and development of nanomedicines employing lipid nanoparticle (LNP) technology for cancer therapies and gene therapies. This work has contributed to five drugs that have been approved by regulatory agencies in the U.S., Europe and Canada. Dr. Cullis has co-founded ten biotechnology companies that now employ over 300 people, has published over 350 scientific articles and is an inventor on over 60 patents. He also co-founded the Centre for Drug Research and Development, a Centre of Excellence for the Commercialization of Research (now AdMare) in 2004, the Personalized Medicine Initiative (PMI) in 2012 and the NanoMedicines Innovation Network in 2019. Dr. Cullis was elected a Fellow of the Royal Society of Canada in 2004 and was also awarded the Prix Galien, Canada's premier prize for achievements in pharmaceutical R&D, in 2011. Two recently approved drugs that are enabled by LNP delivery systems devised by Dr. Cullis, members of his UBC laboratory and colleagues in the companies he has co-founded deserve special emphasis. The first is Onpattro which was approved by the US FDA in August 2018 to treat the previously fatal hereditary condition transthyretin-induced amyloidosis (hATTR). Onpattro is the first RNAi drug to receive regulatory approval. The second is BNT162b2, the COVID-19 vaccine developed by Pfizer/BioNTech that has now (December 2020) been approved in Canada, the USA, the UK and Europe. It is anticipated that more than 1.3B doses of BNT162b2 will be administered worldwide in 2021.

Title: **Lipid Nanoparticle (LNP) Systems for Enabling Gene Therapies: Applications for an Hereditary Disease and a COVID-19 Vaccine**

Abstract: Gene therapies employing genetic drugs such as small interfering RNA (siRNA) for gene silencing and mRNA for gene expression have the potential to cure most diseases. However, sophisticated delivery systems are required to enable clinical use of nucleic acid polymers as they are readily broken down in biological fluids, do not accumulate at sites of disease and cannot penetrate target cells even if they arrive at target tissues. Lipid nanoparticle (LNP) technology is increasingly enabling the clinical potential of genetic drugs by packaging the nucleic acid polymer in well-defined nanoparticles that protect the nucleic acid payload in vivo and facilitate intracellular delivery following uptake into target cells by endocytosis. This approach has received clinical validation with the approval of Onpattro by the FDA in 2018. Onpattro consists of an LNP containing siRNA to silence transthyretin in hepatocytes, thereby arresting and reversing the disease transthyretin induced amyloidosis (hATTR), a disease that was previously untreatable and was fatal within five years of diagnosis. In this talk I will describe the design features that were followed to develop Onpattro and how related technology is being employed to enable mRNA-based drugs. A notable example is the development of LNP mRNA vaccines, which are playing a leading role in the global response to Covid-19.