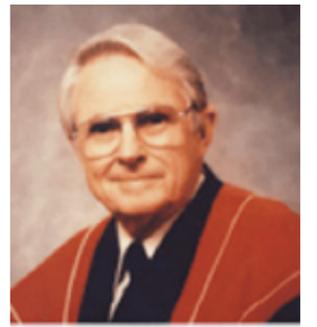




THE UNIVERSITY OF BRITISH COLUMBIA
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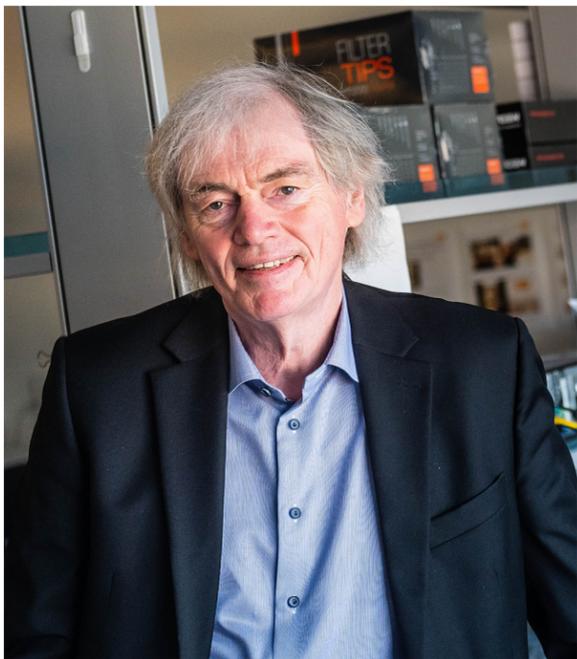
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Funded by an endowment from the
Canadian Institutes of Health Research
and the Wolfe & Gita Churg Foundation

Faculty of Medicine
Department of Cellular & Physiological Sciences

THE 19th D. HAROLD COPP LECTURE

Friday, May 5th, 2023 at 3:00 pm Life Sciences Centre (LSC 2)
2350 Health Sciences Mall, Room 1002



Pieter R. Cullis, Ph.D.

Professor, Dept of Biochemistry and Molecular Biology,
Department of Botany
Life Sciences Centre, University of British Columbia

Founding Director, Centre for Drug Research and
Development

Founding Scientific Director, NanoMedicines
Innovation Network

Lipid Nanoparticle Systems for Enabling Gene Therapies

Gene therapies employing genetic drugs such as small interfering RNA (siRNA) for gene silencing and messenger RNA (mRNA) for gene expression have the potential to cure most diseases. However, sophisticated delivery systems are required to enable the therapeutic use of nucleic acid polymers as they are quickly broken down in biological fluids, do not accumulate at sites of disease and cannot penetrate into 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 in well-defined nanoparticles that protect the payload following systemic in vivo administration and facilitate intracellular delivery following uptake into target cells by endocytosis. Clinical validation of this approach was achieved by the approval of Onpattro by the US FDA in 2018 to treat the disease transthyretin-induced amyloidosis (hATTR). Onpattro consists of LNPs containing siRNA that silences production of transthyretin in the liver (in hepatocytes) following intravenous administration. In this talk I will describe the historical development of LNP systems leading to the development of Onpattro and how related LNP delivery technology is being employed to enable many mRNA-based gene therapy drugs. A notable example of the success of this approach is the development of the Pfizer/BioNTech COVID-19 mRNA vaccine, which has played a leading role in alleviating the Covid-19 pandemic.

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