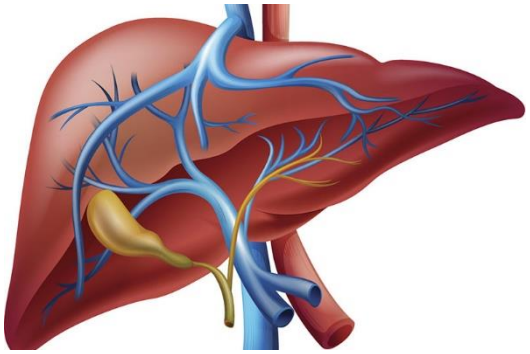
Ian E. Alexander - Professor in Paediatrics and Molecular Medicine

Will give a lecture

The liver as a gene therapy target; exciting progress and prospects!



In this talk, Prof Alexander will provide an overview of the current status and trajectory of the gene therapy field, placing contemporary progress in historical context.

Monday, October the 29th 09:30

Samodzielny Publiczny Dziecięcy Szpital Kliniczny im. Józefa Polikarpa Brudzińskiego w Warszawie

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Ian E. Alexander - Professor in Paediatrics and Molecular Medicine - BMedSci, MBBS (Hons), PhD, FRACP (paeds), HGSACG, FAHMS

Professor Alexander is head of the Gene Therapy Research Unit, a joint initiative of Sydney Children's Hospitals Network and Children's Medical Research Institute in Sydney. Within the hospital he also holds appointments as a senior staff specialist and Director of laboratory research. His training and day-to-day activities in both clinical medicine and laboratory research reflect his interest in translating research progress into improved health outcomes for children. After finishing specialty training in paediatrics he obtained a PhD in Molecular Biology from the Garvan Institute in Sydney before completing clinical genetics training at the Murdoch Institute in Melbourne. He then undertook postdoctoral studies at the Fred Hutchinson Cancer Research Center in Seattle, returning to Australia to take up his current position. His specific expertise and interests include virus-mediated gene transfer with a focus on metabolic liver disease and primary immunodeficiencies. He is currently a member of the International Committee of the American Society of Gene and Cell Therapy, Associate Editor for *Human Gene Therapy* and *The Journal of Gene Medicine* and an Editorial Board Member for *Molecular Therapy - Methods & Clinical Development*.

The liver as a gene therapy target; exciting progress and prospects!

In this talk, Prof Alexander will provide an overview of the current status and trajectory of the gene therapy field, placing contemporary progress in historical context. Emphasis will be given to key enabling technologies and advances that are progressively bringing more disease phenotypes within therapeutic reach. Vectors based on recombinant adeno-associated virus (rAAV) will be given special attention in light of recent therapeutic successes using this system to treat diseases of the eye, central nervous system and liver. Progress and prospects in liver-targeted gene therapy by both gene addition and genome editing technologies will be explored in detail. This will include examination of relevant aspects of liver biology and dissection of the differing challenges posed by specific target diseases.