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CIOMS Working Group XIII consensus report on *Real-world data and real-world evidence in regulatory decision making*

23 January 2025

2 pm – 3.30 pm* CET

*Corresponds to 8 am US Eastern Standard Time, and 10 pm Japan Standard Time

Biographical notes

Lembit Rägo
Secretary-General,
CIOMS, Switzerland

Lembit Rägo, MD, PhD, was a Professor of Clinical Pharmacology (Tartu University, Estonia) and founder and first Director General of the Estonian Drug Regulatory Authority, State Agency of Medicines. In December 1999, he joined the World Health Organization (WHO) Headquarters, Geneva, as Coordinator of Quality Assurance and Safety: Medicines (QSM) team which included activities related to International Nonproprietary Names (INNs), Quality Assurance, Pharmacovigilance, Regulatory Support and Fighting Falsified Medicines. In 2001, Dr Rägo laid the foundations for the WHO Prequalification of Medicines Programme, which he continued to develop until he left WHO. From September 2013, Dr Rägo served as the Head/Director of WHO's Regulation of Medicines and Other Health Technologies unit which first time united all regulatory activities related to medicines, vaccines, biologicals and medical devices into one single entity. Since 2000, he has served as observer to ICH, first representing WHO and now CIOMS.

He is well known to many senior regulators in all parts of the World, frequent speaker in different international fora and has numerous publications including on several aspects of regulatory affairs.

He was appointed Secretary-General of CIOMS in April 2016.

Alar Irs
Chief Medical Officer, State Agency of Medicines, Estonia

Alar Irs is the Chief Medical Officer for the Estonian Drug Regulatory Authority, State Agency of Medicines, being responsible for the Agency's participation at the EU/EMA scientific committees, for the medical quality of the Agency's decisions and for communication with stakeholders. Dr Irs represents Estonia at the European Medicines Agency's (EMA) Committee for Human Medicinal Products (CHMP) and is the Chair of its Cardiovascular Working Party.
Dr Irs has trained as a clinical pharmacologist and as an interventional and intensive care cardiologist. He is Head of Heart Clinic at the Tartu University Hospital and a Senior Teaching Physician in Cardiology at the departments of Angiography and Cardiac Intensive Care.

Juhaeri Juhaeri
Vice President and Global Head, Epidemiology and Benefit-Risk, Sanofi, US

Juhaeri Juhaeri is an epidemiologist with over thirty years of experience in real-world evidence and epidemiology in academia and in the pharmaceutical industry. Currently, he is Vice President and Global Head of Epidemiology and Benefit – Risk at Sanofi. He has implemented new innovative approaches to real-world evidence, signal detection, and structured benefit – risk evaluation, and has built and led various teams in Pharmacovigilance and Patent Safety.

He has contributed to various public-private collaborations between pharmaceutical industry, academia, and regulatory agencies. He was a member of the Editorial Committee of the CIOMS Working Group XIII on "Real-World Data and Real-World Evidence in Regulatory Decision Making". Currently, he is the industry co-lead of the European Innovative Health Initiative Real-World Evidence project to generate, pilot-test, and disseminate evidence-based guidance and tools for the use of real-world evidence to inform the development and evaluation of medicines, medical devices, and drug-device combinations. Prior to that, he was the industry lead of Methods Working Group in the Innovative Medicines Initiative (IMI) project on patient preference in benefit-risk evaluation in the whole drug life cycle.

Yoshiko Atsuta
Scientific Director, Japanese Data Center for Hematopoietic Cell Transplantation (JDCHCT)
and
Professor, Department of Registry Science for Transplant and Cellular Therapy, Aichi Medical University School of Medicine, Japan

Yoshiko Atsuta is the Scientific Director of the Japanese Data Center for Hematopoietic Cell Transplantation (JDCHCT), and Professor of Department of Registry Science for Transplant and Cellular Therapy, Aichi Medical University School of Medicine. The JDCHCT, in collaboration with the Japan Society for Transplantation and Cellular Therapy, perform nationwide survey to collect hematopoietic cell transplantation and cellular therapy outcome data, and to promote patient registry data utilization for research and for regulatory purposes. She is actively involved in international collaboration of the area whose activities include chairing the Registry Committee of the Asia-Pacific Blood and Marrow Transplantation Group, and chairing Accreditation Committee of the Worldwide Network of Blood and Marrow Transplantation.

David Townend

Professor of Health and Life Sciences Law and Associate Dean (Research and Enterprise), The City Law School, City St George's, University of London, UK

David Townend is Professor of Health and Life Sciences Law and Associate Dean (Research and Enterprise) at The City Law School, City St George's, University of London, UK. He works in the Socio-legal Studies tradition, focussing on: conceptual issues of privacy, property and solidarity in law and ethics; and practical questions relating to data protection and the use of personal data in healthcare and research, and the general regulation of health research.

Sean Hennessy

Professor of Epidemiology and of Systems Pharmacology and Translational Therapeutics / Director, Division of Epidemiology, Perelman School of Medicine, University of Pennsylvania, US

Sean Hennessy uses healthcare data to generate real world evidence about the health effects of prescription drugs. His team has identified a survival benefit of potassium supplementation in users of loop diuretics and studied serious health consequences of drug-drug interactions involving high-risk drugs including anticoagulants, antidiabetes agents and antiplatelet agents. His research has produced crucial knowledge about the cardiovascular safety of many widely-used drugs for mental health conditions including ADHD, depression and schizophrenia. He also evaluated an early approach to using medical insurance data to improve prescribing, finding it ineffective despite its federal mandate. This contributed to the omission of drug utilization review programs from Medicare Part D. He co-led a pair of studies demonstrating the effectiveness and safety of the SA14-14-2 vaccine for Japanese encephalitis (JE), which subsequently led to the immunization of millions of children per year in many populous countries including Cambodia, India, Malaysia, Nepal, Sri Lanka and Thailand. Use of that vaccine has been credited with reducing the incidence of JE. He co-developed the instrumented difference-in-differences design for study of the effects of rapidly increasing or declining exposures.

He was also the senior author of a citizen petition to the U.S. Food and Drug Administration that led to re-labelling of metformin, the best-proven oral drug for diabetes, to permit its use in persons with mild to moderate renal insufficiency.
Dr. Hennessy is a past scientific chair and past president of the International Society for Pharmacoepidemiology, past chair of NIH's Health Services Quality and Effectiveness study section,and has served on the FDA's Drug Safety and Risk Management Advisory Committee and the board of directors of the American Society for Clinical Pharmacology and Therapeutics. He is a co-editor of the books Pharmacoepidemiology, 6th edition and Textbook of Pharmacoepidemiology, Third edition.

Elodie Baumfeld Andre
Head of Real-World Data (RWD) team, Roche Information Solutions, US

Elodie Baumfeld Andre is the Head of Real-World Data (RWD) team at Roche Information Solutions, which supports the entire diagnostic product portfolio, including both in-vitro assays and digital health products, by generating insights and evidence to enable data-driven decision-making.
Previously, she was the Real-World Evidence (RWE) Regulatory Strategy Lead at Pfizer, where she focused on generating RWE to support regulatory decision-making, driving innovation in clinical trial design. In her earlier career, she filled a variety of roles across Medical Affairs and Health Economics & Outcomes Research, strategically supporting and positioning Pfizer's cardiovascular, neuroscience, and pain/inflammation portfolios, and leveraging RWD through observational research studies. Dr..Baumfeld Andre also served as the Chief of Staff to Pfizer's Global President of Worldwide Research & Development, advising and providing operational support for some of the company's key R&D initiatives.
Dr. Baumfeld Andre holds a PhD in Pharmacoepidemiology from the Université de Montréal in Canada, as well as a Master's in Pharmacology and a Bachelor's degree in Physics from UFR Sciences et Techniques in France.

Anja Schiel
Senior Adviser, Norwegian Medical Products Agency (NOMA), Scientific Advice Working Party, European Medicines Agency (EMA) and MPG, and JSC member HTA-Coordination Group (HTA-CG), Norway

Anja Schiel has studied Biology at the Johannes Gutenberg-University, Mainz, Germany. She received her PhD from the Free University in Amsterdam in 2006 and worked several years as Post-Doc on a range of subjects focusing on oncology, immunology and molecular biology, first at the University of Leiden, the Netherlands, and later at the University of Oslo, Norway, before starting at the Norwegian Medicines Agency (NoMA) in 2012.
At NOMA (since January 2024 renamed to Norwegian Medical Products Agency) she is working as Special Adviser/Statistician/Methodologist both on regulatory and Health Technology Assessment (HTA) projects. She has been Chair of EMA's (European Medicines Agency's) Biostatistics Working Party (2017 – 2019) and during 2019 – 2022 she was Chair of EMA's Scientific Advice Working Party (SAWP). She continues currently as alternate member at the SAWP and is member of the recently established Methodology Working Party (MWP) at EMA.
She has been involved in EUnetHTA JA3 and its successor, EUnetHTA 21, with particular focus on parallel EMA-HTA scientific advice (joined scientific consultations). As one of the vice Chairs of the JSC Committee for Scientific Consistency and Quality (JSC CSCQ) she was involved in the preparation of the implementation of developer support under the Regulation on Health Technology Assessment (HTAR, to apply from January 12th 2025). Currently she is a member of the Joined Scientific consultation sub-group (JSC SG) and alternate Member in the Methodological and Procedural sub-group (MPG SG).

David Shaw
Senior Researcher,
Institute for Biomedical Ethics at the University of Basel, Switzerland
and
Associate Professor of Health Ethics & Law,
Maastricht University, the Netherlands

David Shaw is Senior Researcher at the Institute for Biomedical Ethics at the University of Basel, Switzerland, and Associate Professor of Health Ethics & Law at Maastricht University, the Netherlands. He was previously Lecturer in Ethics in the School of Medicine of the University of Glasgow, Scotland, and Research Fellow in Ethics, Philosophy and Public Affairs at the University of St Andrews, Scotland. He is interested in all areas of bioethics, but particularly organ donation and research ethics.