Health Technology Assessment during transition time.
Opportunities and uncertainties

Round table, October 27th, 2021, Online

DEBATE REPORT

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INTERNATIONAL CONTEXT

The European states are actively working on changing, to give more and fairer access to patients to new drugs, devices, diagnostics and digital technologies.

Innovation is transforming healthcare and helping tackling unmet medical needs. New shared solutions are needed to make the most of the disruptions in access and ensure best outcomes for patients and a more equitable and speedy access. There is a need to work on a fair and objective way towards accessibility and innovation.

Moreover, Real-world evidence (RWE) holds enormous promise, with some of that promise beginning to be realized in the evaluation of harms.

Health Policies Journal organised a professional talk with the most representative experts at the European and Romanian level to see what's changing and how this will improve our health and healthcare process.

OCTOBER 27TH EVENT:

- 71 participants (experts and authorities from Romania, Republic of Moldova and Brussels)
- 10 speakers
- 2.30 hours debate

CHAIRMAN:

MEP Cristian Bușoi, Chair of the European Parliament's Committee on Industry and Research, the European Parliament's rapporteur for the EU Independent Health Programme, responsible for EMA on behalf of the European Parliament, Chair of the EP Cancer Intergroup

CO-CHAIRMAN

Dr. Adrian Pană, Health Policy and Health Management Expert

SPEAKERS:

EUROPEAN AUTHORITIES

Mrs. Flora Giorgio, Deputy Head of Unit, B6 Medical Devices and HTA, DG SANTE, European Commission

Mr. Michael Berntgen, Head of Scientific Evidence Generation Department at European Medicines Agency

ROMANIAN AUTHORITIES

Dr. Andrei Baciu, Secretary of State, Romanian Ministry of health

Prof. dr. pharm. Robert Ancuceanu, President of the Romanian National Agency for Medicines and Medical Devices & HTA team

ACADEMIA STAKEHOLDERS

Prof. univ. dr. Florentina Furtunescu, Vice-rector, University of Medicine and Pharmacy „Carol Davila", Faculty of Medicine,Department of Public Health and Management

Dr. Ştefan Strilciuc, MPH, researcher at University of Medicine and Pharmacy "Iuliu Hatieganu" Cluj-Napoca

INDUSTRY STAKEHOLDERS

Mrs. Alina Culcea, President ARPIM (Romanian Association of International Drug Manufacturers)

Mr. Jorge Ruiz Benecke, General Manager, ABBVIE Romania

Dr. Ioana Bianchi, External Affairs Director, ARPIM

Dr. Anca Bundoi, Market Access & Governmental Affairs Director, AstraZeneca

Nona Chiriac, Public Affairs Head Novartis & Patient Access Head, Novartis Pharma Romania

#TOPICS

Would greater collaboration across member states really improve HTA processes?

How EMA is driving the shift towards improving the regulatory decision-making field?

How to prove value and demonstrate solutions to unmet patient need to accelerate approval timelines, empower get treatments into patient hands quicker?

Understanding & maximizing the value of Real-World Evidence (RWE)

Progress in the use of RWE across the value chain as a successful transition to a digitally enabled, value-driven healthcare system

How can RWE give more value to patients through key health authorities?

How advances in analytics, data quality and collection, interoperability and new collaborations are further enabling RWE's value?

How to create an efficient co-collaborative framework which ensures clarity on roles, provides value back to patient groups, and delivers an easier flow of information?

How to improve trial efficiency with novel design approaches, to enable outcomes-based pricing agreements and provide more strategic insights to enhance decision-making across the value chain?

Flora Giorgio, Deputy Head of Unit - B6 Medical Devices and HTA, DG SANTE

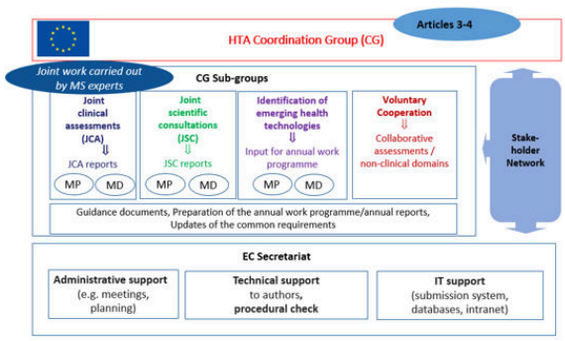
„I congratulate the Romanian authorities for their efforts to strengthen the agency's capacity and, in general, to meet the enormous challenge of facilitating patients' access to innovation. We are confident that the new EU HTA framework can be helpful on both fronts. We will have to start working on this with the EMA, the industries, academia and all the key players to make it a reality. We are confident that, at this preparatory stage, cooperation at EU level could, hopefully, also support national efforts. „

ROUND TABLE - CONCLUSIONS

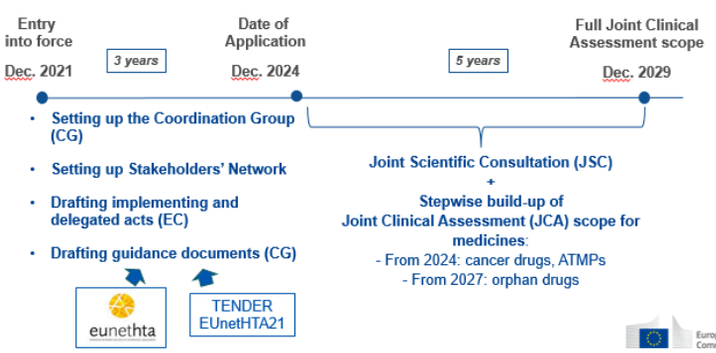
HTA Regulation
Key principles

- **Joint work** on common **scientific, clinical aspects** of HTA
- Joint work **driven by Member State HTA bodies**
- Ensure **high quality, timeliness and transparency**
- Ensure **use of joint work in national HTA processes**
- **Member States** remain responsible for:
  - Drawing **conclusions on added value** for their health system
  - Taking **decisions on pricing & reimbursement**
- **Progressive implementation**

HTA Regulation - Governance



HTA Regulation
Timeline of implementation



HTA Regulation
Next steps (to be confirmed)

- December 2021 – expected date of adoption
- Q1 2022 – call for Member States to nominate their representatives for the Coordination Group
- Mid-2022 – first meeting of the Coordination Group
- Q4 2022 – launch of the procedure for setting up the Stakeholder Network

Mr. Michael Berntgen, Head of Scientific Evidence Generation Department at European Medicines Agency

”At their last bilateral meeting of Joint Action 3, the EMA and EUnetHTA agreed to establish a list of priority areas for future collaboration between regulators and HTAs at European level, with a view to continuing future work. The overall objective of such collaboration is to improve the efficiency and quality of processes, while respecting the responsibilities of different decision-makers, and ensuring mutual understanding and dialogue on evidence needs, in order to facilitate access for patients in the European Union to drugs. With regard to the Real-World Data Analysis and Query Network (DARWIN EU), the aim is to provide timely and reliable evidence on the use, safety and efficacy of medicinal products for human use, including vaccines, from databases in the real world of healthcare across the EU”.

EUROPEAN MEDICINES AGENCY

DRAFT priority areas for future collaboration

Product-specific work	*Methodological work*	*Operational work*
Joint scientific consultation on evidence generation, including PLEG	Study methods and guide-lines of real-world evidence, including for registries	Continuous optimisation of regulatory outputs
Exchange of information on the respective assessments of medicinal products by regulators and HTA bodies	Generation of patient relevant data / information to support decision making	Methodologies for engage-ment of patients and HCPs
Evidence planning and assessment for advanced therapy medicinal products	Extrapolation / evidence transfer as tool to support assessment in smaller populations	Practices in the context of companion diagnostics
		Horizon scanning and preparedness of HTA and regulatory systems

4 European collaboration at the regulatory/HTA interface 27 October 2021

Classified as public by the European Medicines Agency

ROUND TABLE - CONCLUSIONS

MEP Cristian Buşoi, Chair of the European Parliament's Committee on Industry and Research, the European Parliament's rapporteur for the EU Independent Health Programme, responsible for EMA on behalf of the European Parliament, Chair of the EP Cancer Intergroup

"Developers of medical technologies should be able to request an update of the clinical evaluation if additional evidence from clinical practice becomes available, even if such a requirement was not specified in the initial joint clinical evaluation. The methodology, while consistent, should also have a level of flexibility that allows for a fair and appropriate assessment of certain therapies that are currently following innovative clinical development pathways or, on the contrary, have limitations. „

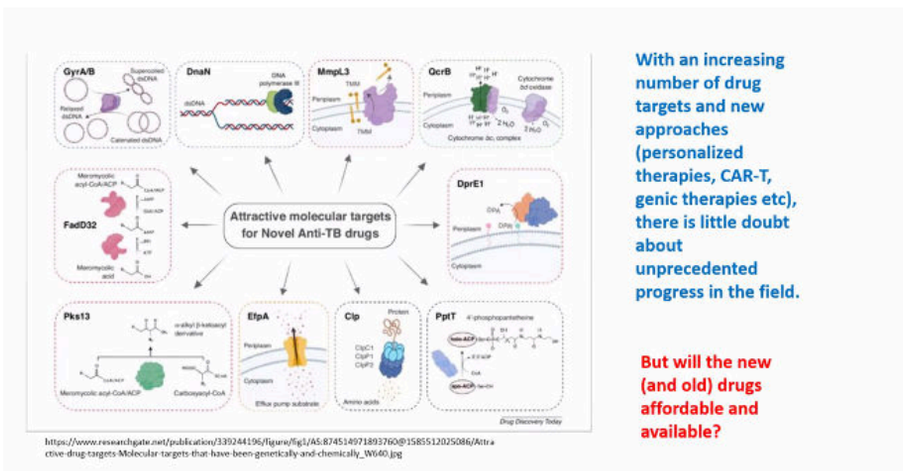
Dr. Andrei Baciu, Secretary of State, Romanian Ministry of health

„The financial challenges we now face, especially caused by the COVID-19 pandemic, are related not only to insufficient revenues, but also to rising health care costs. Regulating the field of medical technology evaluation is one of our priorities, because it is important to find ways to streamline spending and prioritize health policies, especially when we have budgetary constraints. The main goal is to offer Romanian patients access to quality medical services, similar to the services offered to other European citizens ”.

ROUND TABLE - CONCLUSIONS

Prof. dr. pharm. Robert Ancuceanu, President of the Romanian National Agency for Medicines and Medical Devices & HTA team

„We are witnessing a real progress, in recent years, in terms of the pharmaceutical sector - new drugs, new approaches (personalized therapies, CAR-T, gene therapies). The big question, from my point of view, is: will these new drugs (but also the oldest ones) be available and accessible, in terms of price, to patients? (...) I think one of the strengths of the new regulations it will be the joint evaluation, which would facilitate good decision-making in the field of health tehchnology assessment ".



ROUND TABLE - CONCLUSIONS

Prof. univ. dr. Florentina Furtunescu, Vice-rector, University of Medicine and Pharmacy „Carol Davila", Faculty of Medicine, Department of Public Health and Management

"HTA is the solution to solve a distinct part of controversial problem of guarantee in the access to the best alternative for all those in needs, but HTA methods have their limits among which is data quality, data availability and data fragmentation. If data for the clinical trials are more accessible, the data for real world studies are over lacking or been fragmented, or under reliable, unexplored or simply not inaccessible.

Indeed, the major sources of data for real world studies are coming from various sources - the clinical data like health records or data coming from disease registries. All we know, these data are almost complete missing in Romania. Patients generated data like health treatment history or patient reported outcomes. The public data have a limitated level of accessibility. Public health data coming from government sources or from public information system, and these one, again, are in part available. We all know that the situation of data accessibility and data reliability is not very good in our country, but i strongly believe that all the countries are facing these problems".

ROUND TABLE - CONCLUSIONS

Dr. Ştefan Strilciuc, MPH, researcher at University of Medicine and Pharmacy "Iuliu Hatieganu" Cluj-Napoca

"A key message is that having data is very important, but is not enough to be able to produce RW evidence and the outputs. We, as universities, need to educated more of our students to be able to handle issues like causality or bias control. Relate to RWD for regulatory decision makers, the objective is appropriate use of valid RWE for regulatory purposes (like safety, efficacy, benefit-risk monitoring). Features of evidence: derived from data source of demonstrated good quality,valid (internal and external validity), consistent (across countries/data sources), adequate (precision, adequate range of characteristics, dose and duration of treatment). The core issue here is being able to draw casual inferrable from these type of data".

Key messages/questions

1. **RW data** essential but **not enough** to produce **RW evidence**
2. Producing useful **RWE** is **a difficult task** – use and development of novel methods for controlling for bias must be considered
3. Attempt at **program evaluation** at the **RO National Health Insurance House**
4. **Role of universities** in aligning **capacity building** efforts with new scientific gold standards and in **enabling reform**

ROUND TABLE - CONCLUSIONS

Real World Evidence

- The potential for RWE is probably greatest in oncology. Effectively implemented, RWE can accelerate the pace of discovery and the impact of new cancer therapies on patients.

- Using robust genomic sequencing data and longitudinal clinical data, RWE analyzes can identify response biomarkers and therapeutic resistance to optimize a drug development strategy.

- The targeted use of RWE derived from electronic health records (EHR) supports the design and optimization of clinical trials.

Mrs. Alina Culcea, ARPIM President

”There are many factors that contribute to the shortcomings in the system: insufficient budget for implementing decisions (according to a PwC study conducted in Romania, per capita health expenditures were 100 euros in 2009 – one third of the European average), the pricing process and refund, the price factor. I think the lesson of the pandemic was clear to us - without innovation we could not have been able to stop the spread of the pandemic. As such, we need to focus more on how to encourage innovation and, above all, how to make it available to patients as soon as possible. „

ROUND TABLE - CONCLUSIONS

Mr. Jorge Ruiz Benecke, General Manager, ABBVIE Romania

”We applaud the progress made in recent years in accelerating evaluations of new medical technologies. At the same time, we believe that these efforts must be sustained, as patients in Romania remain the last in Europe to have access to innovation, after almost 2 and a half years since the approval of the European Medicines Agency. In this regard, we emphasize the need for collaboration to overcome obstacles to patients’ access to innovative medicines designed to improve health and, in many cases, save lives. The responsibility of treating patients is ours and we need solidarity and collaboration to find solutions together for the benefit of patients”.

Dr. Ioana Bianchi, External Affairs Director, ARPIM

”Regarding the uncertainties of the RWE methodology for heatlh technology assement in Romania, it is worth mentioning that the implementation norms are still missing, thus being only a complementary mechanism. Are there also discussions about what notification versus decision means? What if the study approval decision is issued at the same time as the reimbursement decision? In other states, with other institutions on hypertension, there is the possibility of dialogue with national drug agencies on the study protocol, for its calibration, which is currently missing from our legislation”.

ROUND TABLE - CONCLUSIONS

RWE IN ROMANIAN HTA METHODOLOGY

- Substitute for the 3 international HTA reports – in table 4 and 7 – 45 points
- Refers to non-interventional studies
- Proof of notification BUT study protocol will be analyzed by HTA and Clinical Trials departments, who can invite for consultations representatives of Specialty Committees from MoH and CNAS representatives
- The decision for the validation of the study protocol will be delivered in max. 3 months from the submission of the study protocol
- The study will take place after the inclusion of the medicine in the reimbursement system

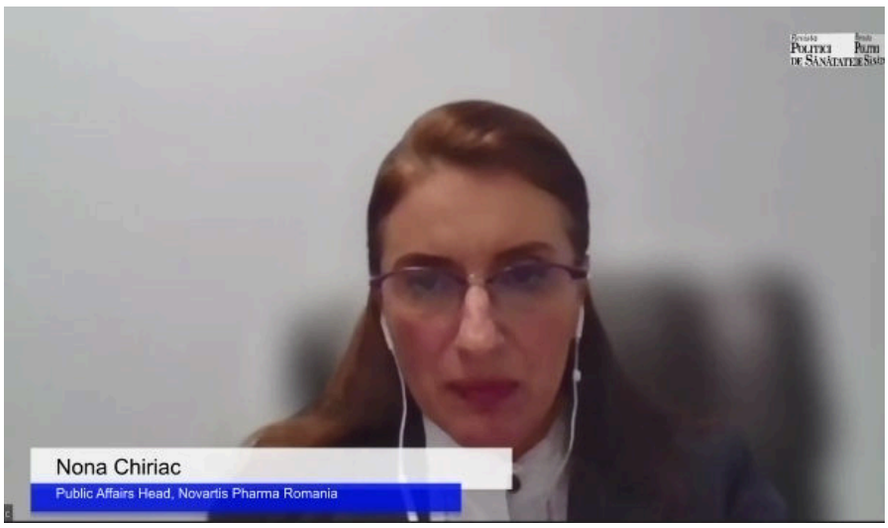
NEXT STEPS

- ARPIM is working on proposals to operationalize the legislative framework
- Methodological guide for conducting RWE studies
- Enlarging the definition of studies
- Use of data collected in the RWE studies
- For health economics : for hospital costs there is a project (CaPESS COST) for re-assessing the DRG tarrifs in public hospitals

Dr. Anca Bundoi, Market Access & Governmental Affairs Director, AstraZeneca

”Regulators are developing approaches to incorporate real-world evidence (RWE) into decision-making. When it comes to market access, RWE improves the evidence on economic value by demonstrating value to payers, comparing data with data from clinical trials to strengthen argumentation, enabling results-based compensation methods, or bringing better / faster access to payers. patients. The proposal for an EU regulation on HTA considers that voluntary cooperation should benefit from research results, such as those on the methods of using real world evidence”.

ROUND TABLE - CONCLUSIONS



Nona Chiriac, Public Affairs Head Novartis & Patient Access Head, Novartis Pharma Romania

”We are aware that we have improvements and learnings across the process. nevertheless, I think the HTA assignment is only a technical step, very important, a major one, but a technical step in a very complex process of securing reimbursements for the proper drugs for the right patients. Because, in the end, it is about having resources, about having a political will and right health political objectives actually to decide which way do we want to go, what we want to prioritize afterwards.We can say that the negative impact is not a criterion for faster access, neither the lack of therapeutic alternatives is not correlated with faster access, and assessment of a new indication follows the same process and timing as per a new International Nonproprietary Names (INN)”

THANK YOU!

HEALTH POLICIES JOURNAL