International Society for Pharmacoeconomics and Outcomes Research

ISPOR – PRIORITIES IN THE AREA OF HEALTH ECONOMICS AND OUTCOME RESEARCH

Prof. Guenka Petrova, MPharm, MEcon, PhD, DSci

MEDICAL UNIVERSITY OF SOFIA, FACULTY OF PHARMACY

PRESIDENT OF ISPOR CENTRAL AND EAST EUROPEAN CONSORTIA

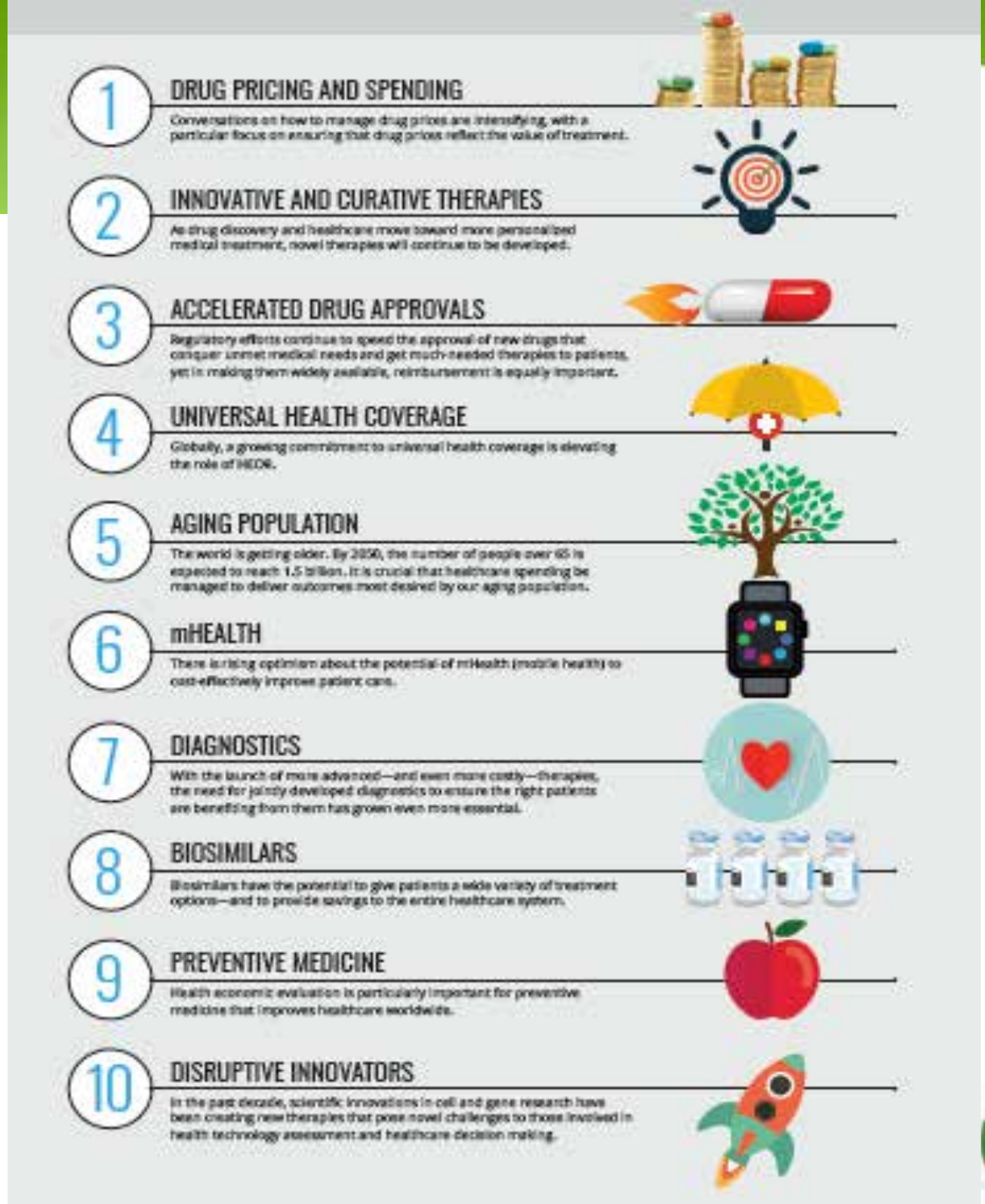
PRESIDENT OF ISPOR BULGARIAN CHAPTER

TOP 10 HEOR TRENDS

Developed by **ISPOR Health Science Policy Council**

Methodology – Comprehensive exploration and collation of potential trends, careful review and vetting of the topics by the council; in-depth survey of ISPOR leaders; feedback and ranking; final discussion and selection at the 20th Annual European congress in November 2017.

RANKING


1
DRUG PRICING AND SPENDING
Conversations on how to manage drug prices are intensifying, with a particular focus on ensuring that drug prices reflect the value of treatment.
2
INNOVATIVE AND CURATIVE THERAPIES
As drug discovery and healthcare move toward more personalized medical treatment, novel therapies will continue to be developed.
3
ACCELERATED DRUG APPROVALS
Regulatory efforts continue to speed the approval of new drugs that conquer unmet medical needs and get much-needed therapies to patients, yet in making them widely available, reimbursement is equally important.
4
UNIVERSAL HEALTH COVERAGE
Globally, a growing commitment to universal health coverage is elevating the role of HTA.
5
AGING POPULATION
The world is getting older. By 2050, the number of people over 65 is expected to reach 1.5 billion. It is crucial that healthcare spending be managed to deliver outcomes most desired by our aging population.
6
mHEALTH
There is rising optimism about the potential of mHealth (mobile health) to cost-effectively improve patient care.
7
DIAGNOSTICS
With the launch of more advanced—and even more costly—therapies, the need for jointly developed diagnostics to ensure the right patients are benefiting from them has grown even more essential.
8
BIOSIMILARS
Biosimilars have the potential to give patients a wide variety of treatment options—and to provide savings to the entire healthcare system.
9
PREVENTIVE MEDICINE
Health economic evaluation is particularly important for preventive medicine that improves healthcare worldwide.
10
DISRUPTIVE INNOVATORS
In the past decade, scientific innovations in cell and gene research have been creating new therapies that pose novel challenges to those involved in health technology assessment and healthcare decision making.

DRUG PRICING AND SPENDING
Balancing value and cost

- Increasing affordability at patient level and maintain cost rising;
- The way of controlling medicines prices is moving towards the calibrating the prices with the value received from treatment;
- Various approaches to value-based pricing are being explored:
  - Performance based (or outcomes based risk sharing agreements) – how much the manufacturer is paid depends on weather, or how well the drug works;
  - Indication specific pricing – to adjust pricing to value when the drug has both high-value and low-value indication;
  - Better measurement of some elements of value not normally captured in cost-effectiveness analysis.
- **In 2018, we should expect to see an extended set of research, process, and policy efforts in this area.**

INNOVATIVE AND CURATIVE THERAPIES – transformative therapies add to budget pressure

- **Transforming the treatment and a life of patients** in a number of disease states (HCV, oncology etc.);
- Drug discovery and health care **moves toward more personalized medical** treatment and curative therapies for chronic and genetic diseases;
- **Tend to be more expensive**, increase budget pressure and threatened sustainability of innovation;
- **Require additional resources for dispensing**, storage, application etc.
- **Collaborative scientific and stakeholders efforts are needed to evaluate and value innovation.**

ACCELERATED DRUG APPROVAL – evidence needs for reimbursement

- **Regulatory efforts to expedite approval of new indications for unmet medical needs**. A balance between faster market access and safety studies is necessary.
  - FDA (2012) – procedures for "accelerated approval" pathway; "breakthrough product" designation;
  - EMA (2014) – "adaptive pathways" as compliment to conditional approval;
  - Japan (2015) – fast-track development and review system;
- **Reimbursement is questionable** – payers are not provided with sufficient evidences of value based clinical outcomes;
- This result **in tension between expedited approval and rapid reimbursement**;
- **Establishment of drug prices in this situation** – outcome based risk sharing agreement, coverage with evidence development;
- **Research needed in the reliability of surrogate markers, use of real world evidences, clinical trials for gathering real world data.**

UNIVERSAL HEALTH COVERAGE – who sets the stage

- UHC is a norm in Europe and many other countries but not in all.
- WHO is a main proponent in existing UHC after the World Health Assembly in 2011;
- ISPOR supports the scientific and educational basis for health technology assessments, particularly with its focus on health economics and outcomes research methodology.
- **With 84 regional chapters, ISPOR and its members around the world are involved in research that can contribute to more informed UHC decision making at the country level.**

AGING POPULATION – controlling rising costs while maintaining quality

- World Health Organization's (WHO) 2010 report, "Global Health and Aging," - 524 million people (about 8% of the world's population) were aged 65 years or older. By 2050, this number is expected to nearly triple - 1.5 billion (16%) of the world's population.
- According to WHO, population aging is likely to influence healthcare spending patterns in developed and developing countries in the decades to come.
- **Health economics and outcomes research can contribute to more effective healthcare spending as the population ages.** Improved measurement and use of individual patient preferences can help ensure that healthcare services are employed pursuing outcomes that bring the most value to patients; this may apply especially to end-of-life care.

mHEALTH – health apps and the rise real-world data

- **Mobile health (mHealth)-generate real-world data, useful for evaluation of health care.**
- **Provide reach but less systematic data**, may or not may be integrated with other health data, can be influenced by selective patients and not generalizable;
- Learning how to make better use of real-world data is crucial to sound evaluation in this area. For all these reasons, **ISPOR and the International Society for Pharmacoepidemiology established a special joint task force on Real-World Evidence in Healthcare Decision Making Initiative** to improve standards and practice for the conduct and reporting of real-world data studies.
- As with drugs and other healthcare products and services, **evidence about the effectiveness, safety, and cost-effectiveness of mHealth is likely to be desired** by clinicians, patients, and payers as they consider adoption and reimbursement of these new technologies.

DIAGNOSTICS – the potential for companion and complementary diagnostics

- **Personalized medicines needs more reliable diagnostic to ensure the appropriate patients are benefiting.**
- Diagnostics market is projected to reach **$6.51 billion by 2022 from $2.61 billion in 2017, at a compound annual growth rate of 20.1%.15 While diagnostics represent just 2% of healthcare costs, they influence 70% of healthcare decisions, and there are $900 million** in estimated total avoidable healthcare costs per year related to lack of appropriate diagnostics use.
- **ISPOR's Medical Devices and Diagnostics Special Interest Group** - explore related to health economics and outcomes research and health technology assessment (HTA) for devices and diagnostics.
- Published work discussing practices **- challenges, and recommendations for HTA of molecular diagnostics.**

BIOSIMILARS – determining the value

- **Regulatory requirements for** biosimilars in the European Union, United States, Latin America, and Asia-Pacific regions are similar **and yet slightly different**.
- According to the *Generic and Biosimilars Initiative Journal*, the **use of biosimilars is expected to result in overall savings from €11.8 billion and €33.4 billion** between 2007 and 2020.
- **Biosimilars have the potential not only to provide cost savings to the healthcare** system but also to give patients a wider set of treatment options.
- However, **costs of switching**, potential differences between the original and the biosimilar, pricing considerations around the appropriate discounts for both original and biosimilar products, and the potential for utilization by more patients given lower biosimilar prices (although not as low in absolute terms as small molecule generics) must also be taken into account.

PREVENTIVE MEDICINE – the promise of improving health through prevention of diseases

- World Health Organization (WHO) estimated that **35 million people would die of chronic diseases such as diabetes, heart disease, chronic respiratory diseases, and tuberculosis. Only 20% of these deaths occur in high-income countries, while 80% occur in low- and middle-income countries.**
- Health economic evaluation is particularly important for preventive medicine.
- **Costs are typically incurred in the short-term to a large population**; benefits accrue in the longer term to a smaller population because not all those at risk would have ultimately experienced the disease.
- **Changing patient behavior must be part of the equation.** ISPOR members are engaging in research that follows, and will help to improve, the best methods and strategies for evaluating disease prevention, including the value of patient-centered behavior changes..

DISRUPTIVE INNOVATORS – CRISP, CART T-CELLS, and the value of new science

- **CRISP – a genome editing technique, CART T-cell therapies – type of adoptive cell transfer in which patients own immune cells are collected and used to treat their cancer. Both brings value to health care – create immediate benefit, create new scientific knowledge.**
- In an economic sense, it is important not to under-reward these **fundamental advances in order to maintain the research incentives for such important breakthroughs.**
- Not all incentives are monetary.
- **This value is not straightforward to measure**, particularly for disruptive therapies because all of their **applications may not be immediately foreseen.**
- **Group-based deliberative processes, such as multiple criteria decision analysis, are being considered increasingly as a way to make decisions about new technologies, especially when their costs and/or benefits extend beyond those that are well-measured by standard economic evaluation.**

Guidelines for Good Research Practices - ISPOR

Take home messages

- The future is bright and scientifically encouraging;
- New technologies bring hope but are costly;
- Mutual efforts and experience sharing are necessary .
- Health and Pharmaceoconomics can propose ideas and its introduction in the real world regulatory practice depend on political will and health system maturation.

Thank you for your attention!