TRENDS IN CLINICAL RESEARCH AND CHALLENGES FOR SMALL COUNTRIES IN THE REGION

Accelerating Growth, Driving Innovation

Ján Filakovsky M.D., Ph.D., MBA

“News related to Clinical Trials’ execution in Lithuania” 19. May 2023

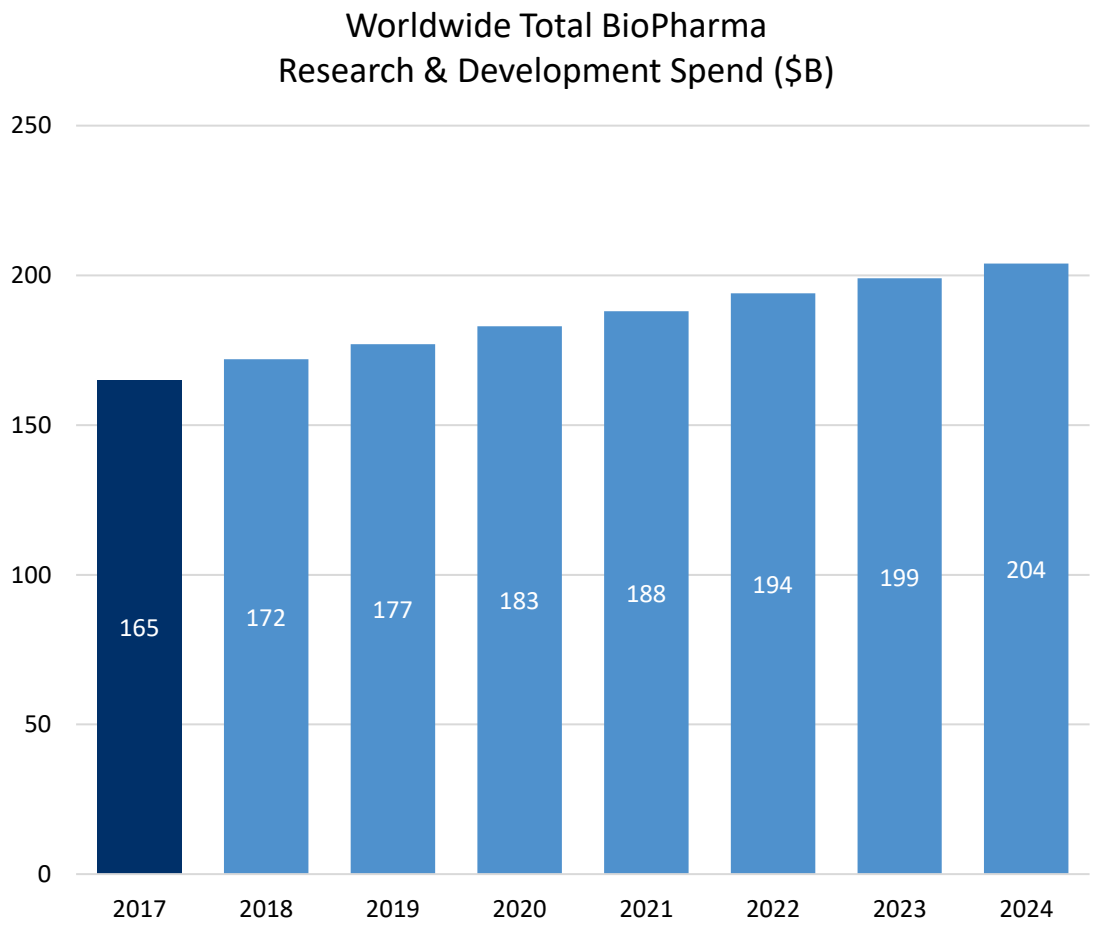
Data is the next frontier

AGENDA

- R&D Growth Trends
- Key Trends Shaping Clinical Research
- The EU Agenda
- Role of Innovation
- Key Opportunities for Small countries

RESEARCH & DEVELOPMENT TRENDS

…and is expected to keep growing



Source: Evaluate Pharma, June 2018; Catalent, Statista; Pharmaprojects 2017

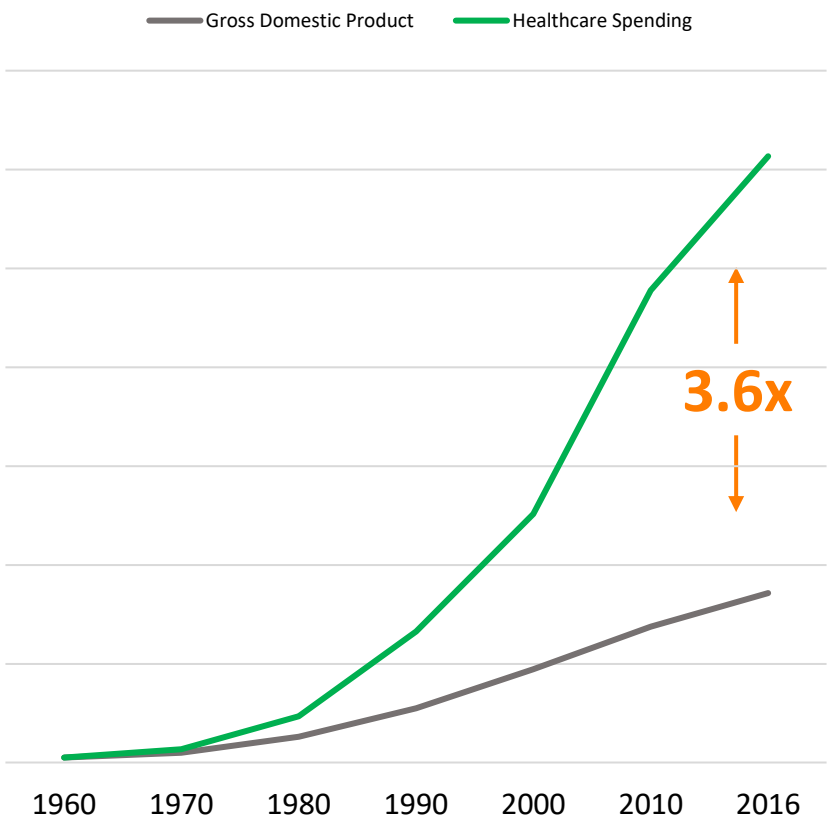
Growth Drivers

- **Growth in Phase III studies.** The number of Phase III studies increased by 7%
- 60% of NDA in Phase I.& II. Development
- **Increasing complexity.** Biologics (products isolated from human, animal, or microorganisms) represent 38% of the R&D pipeline; Oncology products grew 16%, nearly twice the rate of the industry pipeline as a whole
- **Longer duration.** Phase III oncology trials take nearly five years to complete, which is 1.5 years longer than 15 years ago
- **More global.** There has been a shift in clinical trial sites to Eastern Europe, Latin America, Asia, the Middle East, and Africa

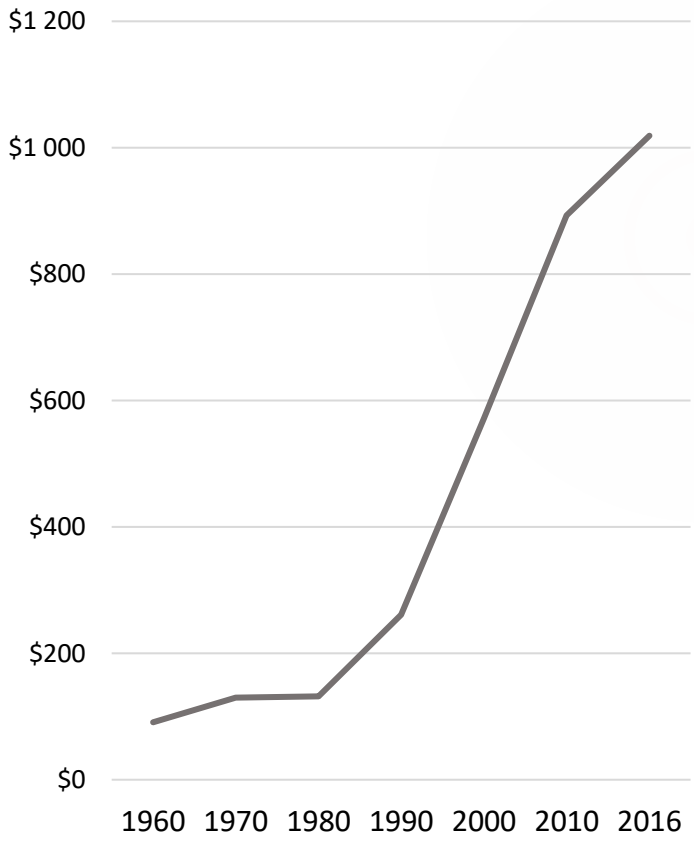
MARKET DRIVERS

The biopharmaceutical industry is driven by waves of spending

Growth of Per Capita Healthcare Spending versus GDP



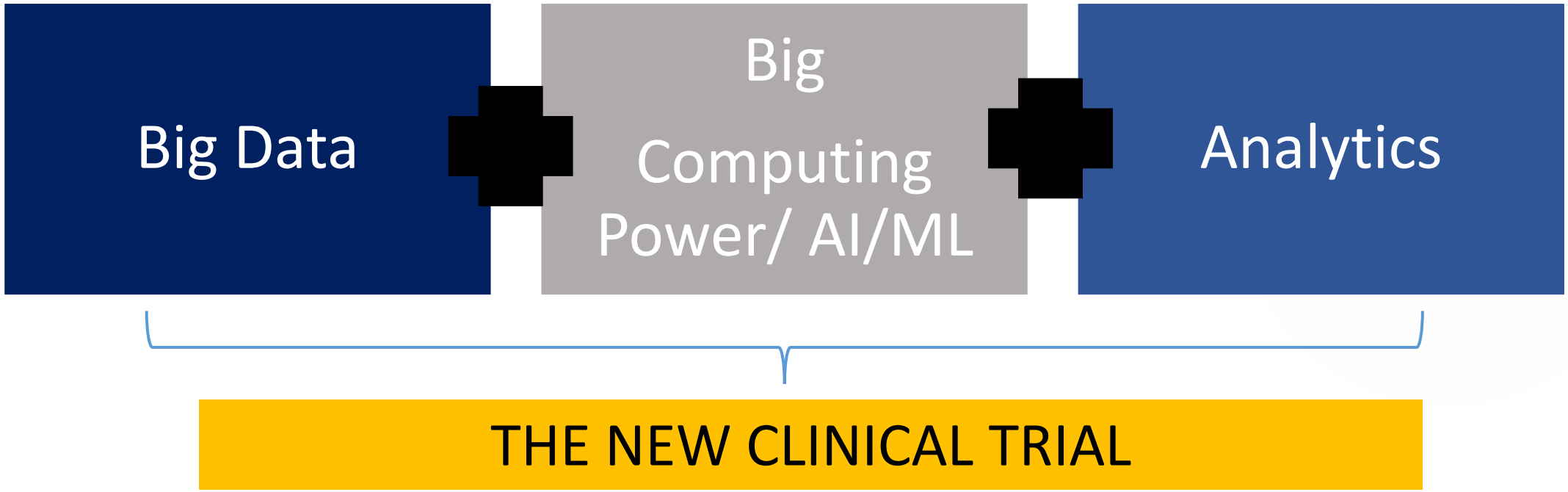
Per Capita Pharmaceutical Spending



Source: Centers for Medicare & Medicaid Services; Peterson-Kaiser

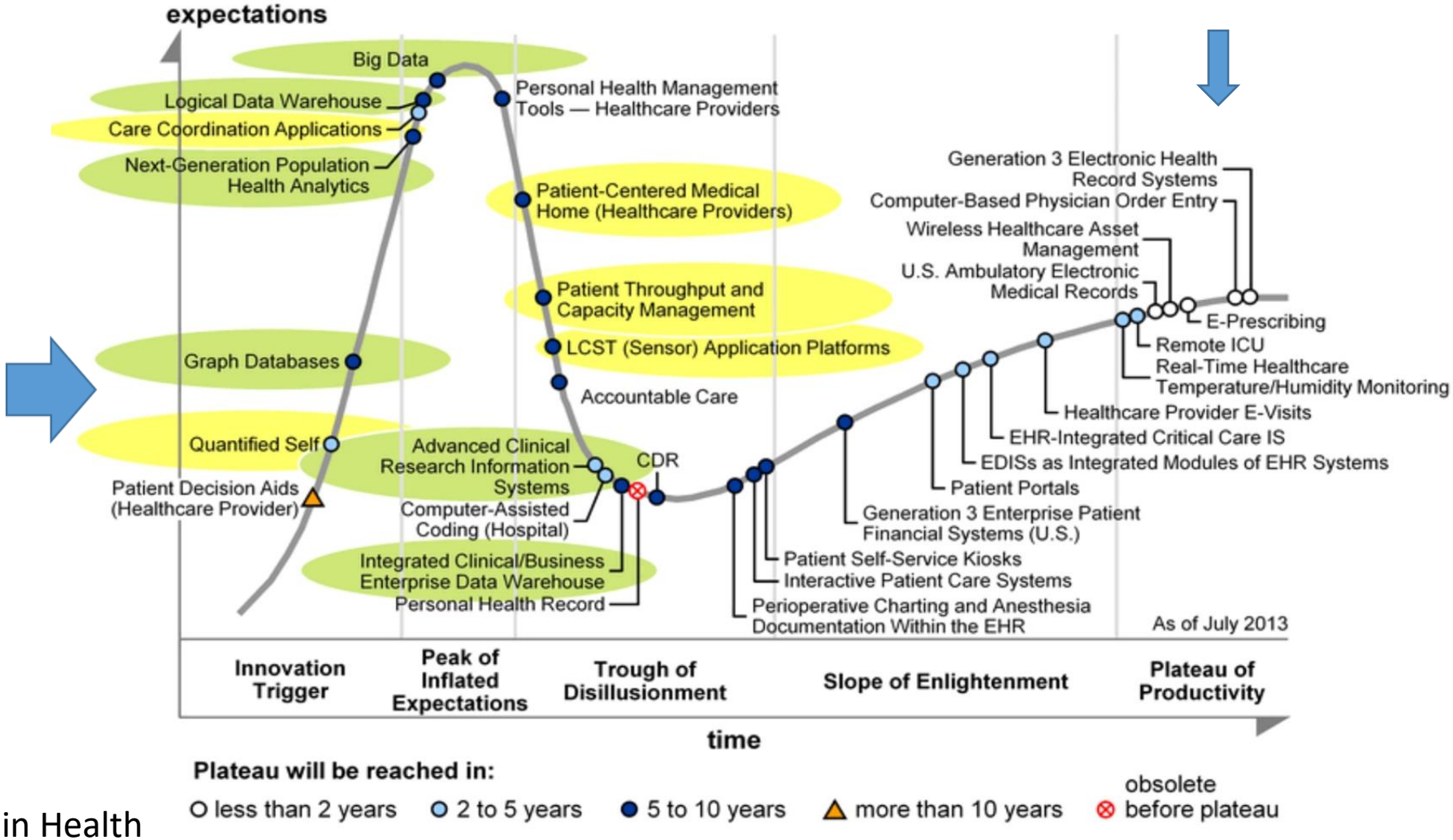
NovaQuest Capital Management

PARADIGM SHIFT



- DATA - THE NEW CURRENCY
- PREDICTIVE ANALYTICS - THE NEW KNOWLEDGE
- SOCIAL MEDIA - THE NEW CHANNELS

COVID-19 “the new norm”
“the accelerator”



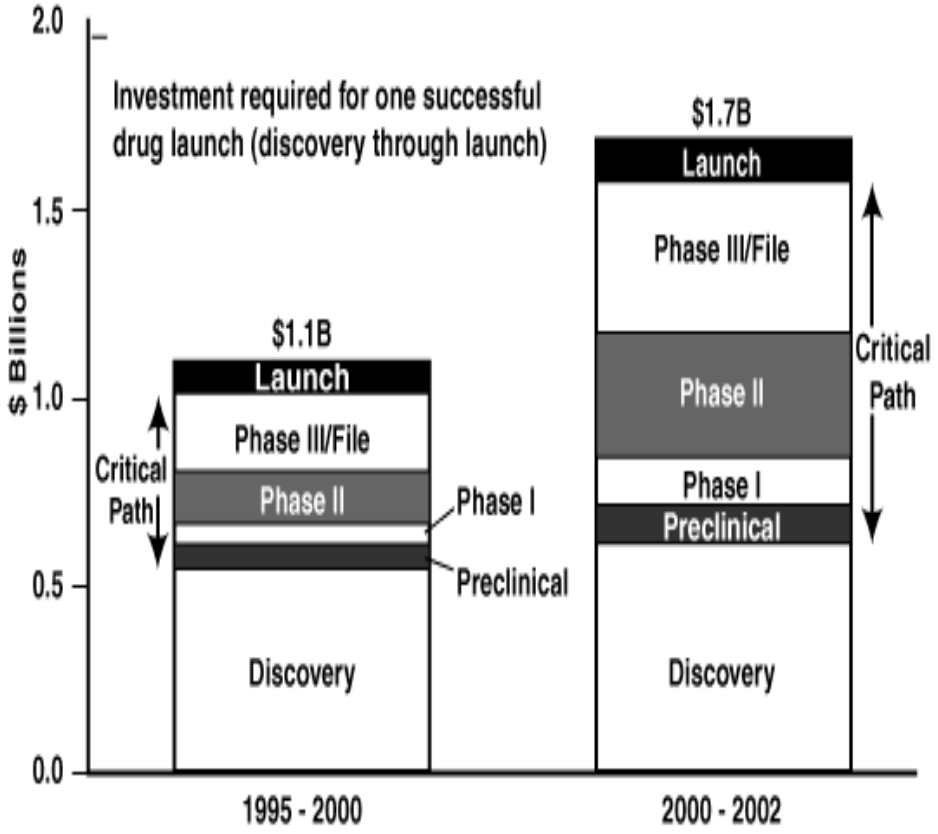
Gartner Hype Cycle: Technology in Health

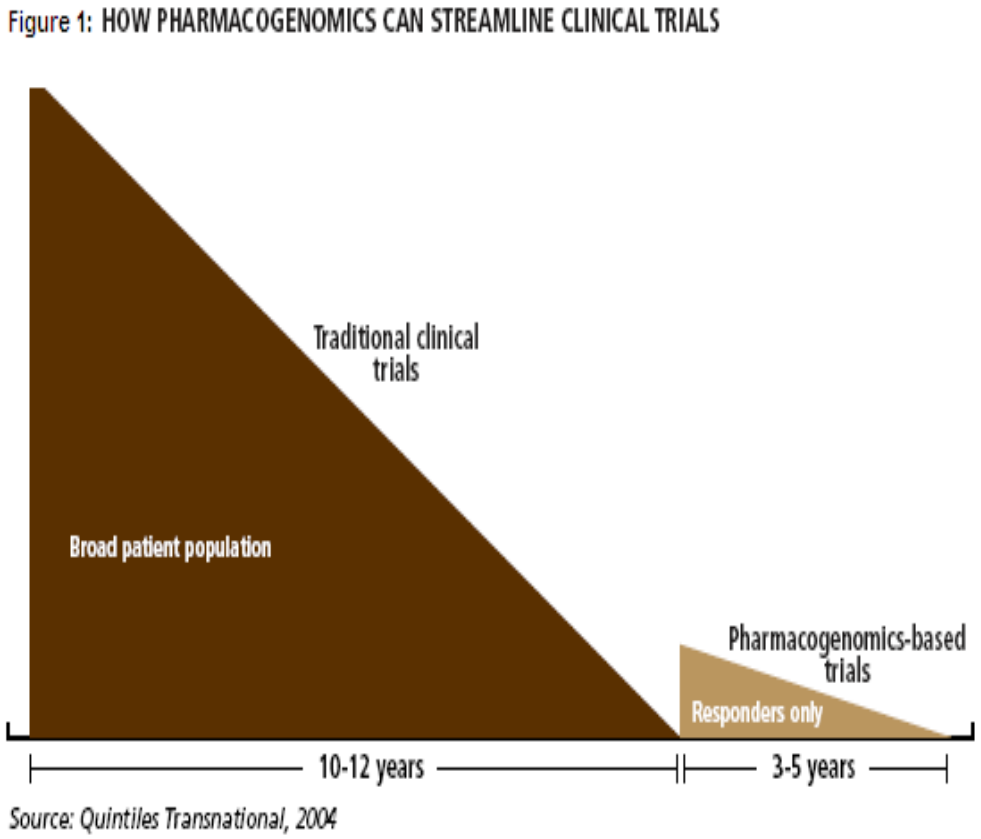
KEY TRENDS SHAPING CLINICAL RESEARCH

- Decentralized Clinical Trials DCTs
- Real- World Evidence
- Precision Medicine
- Artificial Intelligence (AI) and Machine Learning (ML)
- Patient-Centric Approaches
- Adaptive Trial Designs
- Regulatory Innovations
- Data Sharing and Collaboration
- Diversity

Accelerating Clinical Development

CHALLENGE AND OPPORTUNITIES





Innovation or Stagnation? Challenge and Opportunity on the Critical Path to New Medical Products. FDA, 2004.

Innovation or Stagnation? Challenge and Opportunity on the Critical Path to New Medical Products. FDA, 2004 In February 2012.

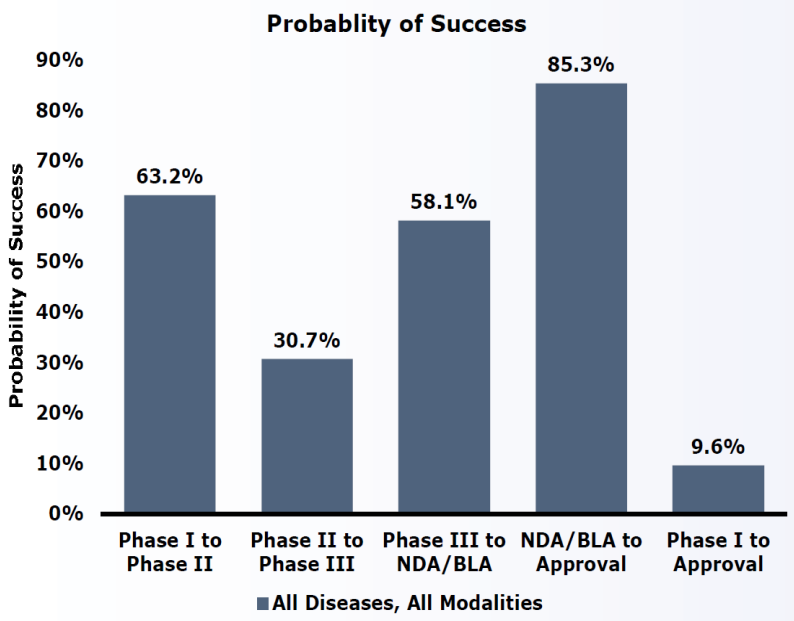
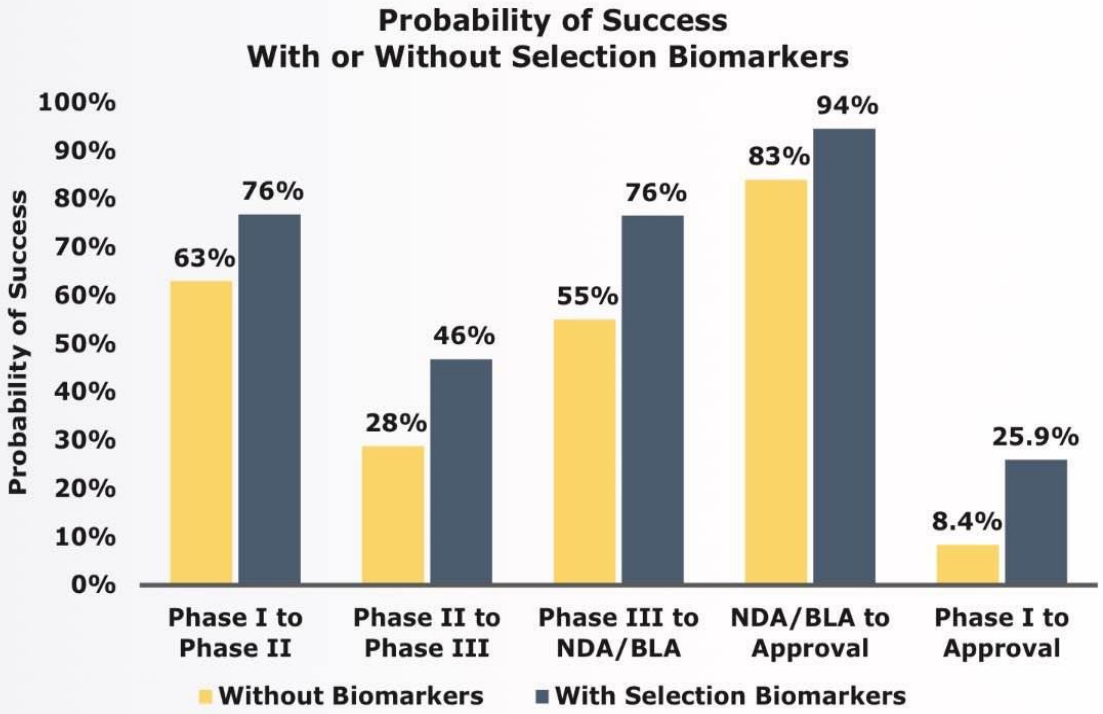


Figure 1. Phase transition success rates and LOA from Phase I for all diseases, all modalities.



- Phase success rate from Phase 1. to LOA (Likehood of Approval)(2003-2011 /BioMedTracker 4451 Drug, 7372 Clinical Development Paths 835 companies,5820 Phase transitions)

  Michael Hay et Al. Nature Biotechnology January 2014

- Based 0n 512 phase transitions out of 9985 (5%) incorporated biomarkers.Phase success rate from Phase 1. to LOA (Likehood of Approval)(2003-2011 /BioMedTracker)

  Clinical Development Success Rates 2006-2015 BIO International Convention , Bio Med Tracker David W Thomas ,Michael Hay et al. 2016

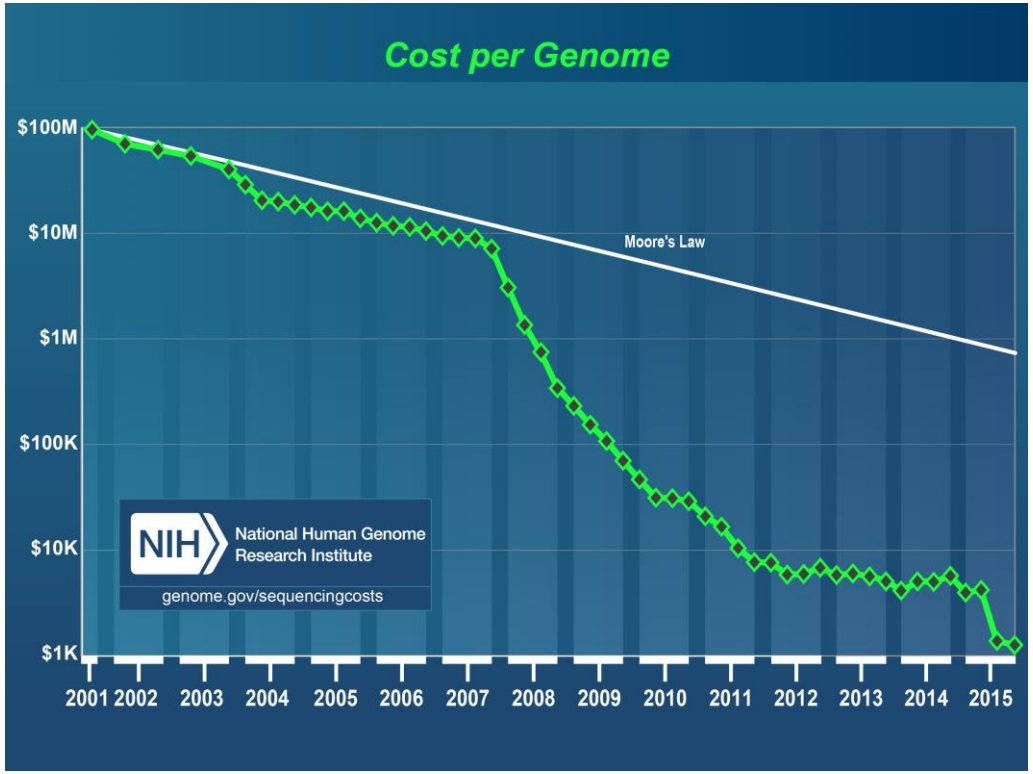
CASE STUDIES

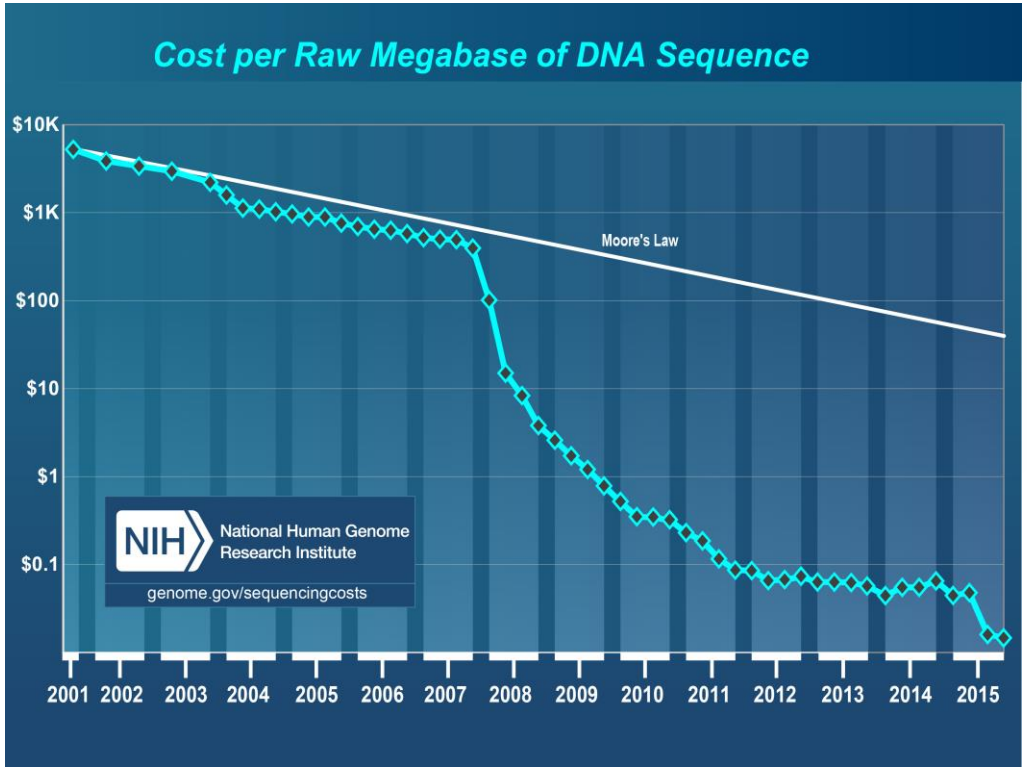
	Cost of CT (% standard költság-beteg)	Subjkects No.	Development Time From PH I. To NDA Submission
Xalkori*	100%	960	1.8**
Iressa*	146%	2850	7.0
Tarceva*	154%	3110	5.3

*Xalkori 2011/2012, Iressa 2003/2005, Tarceva 2004/2012
** only phase 2 trial results for FDA approval. EMA approval included interim phase 3 trial results

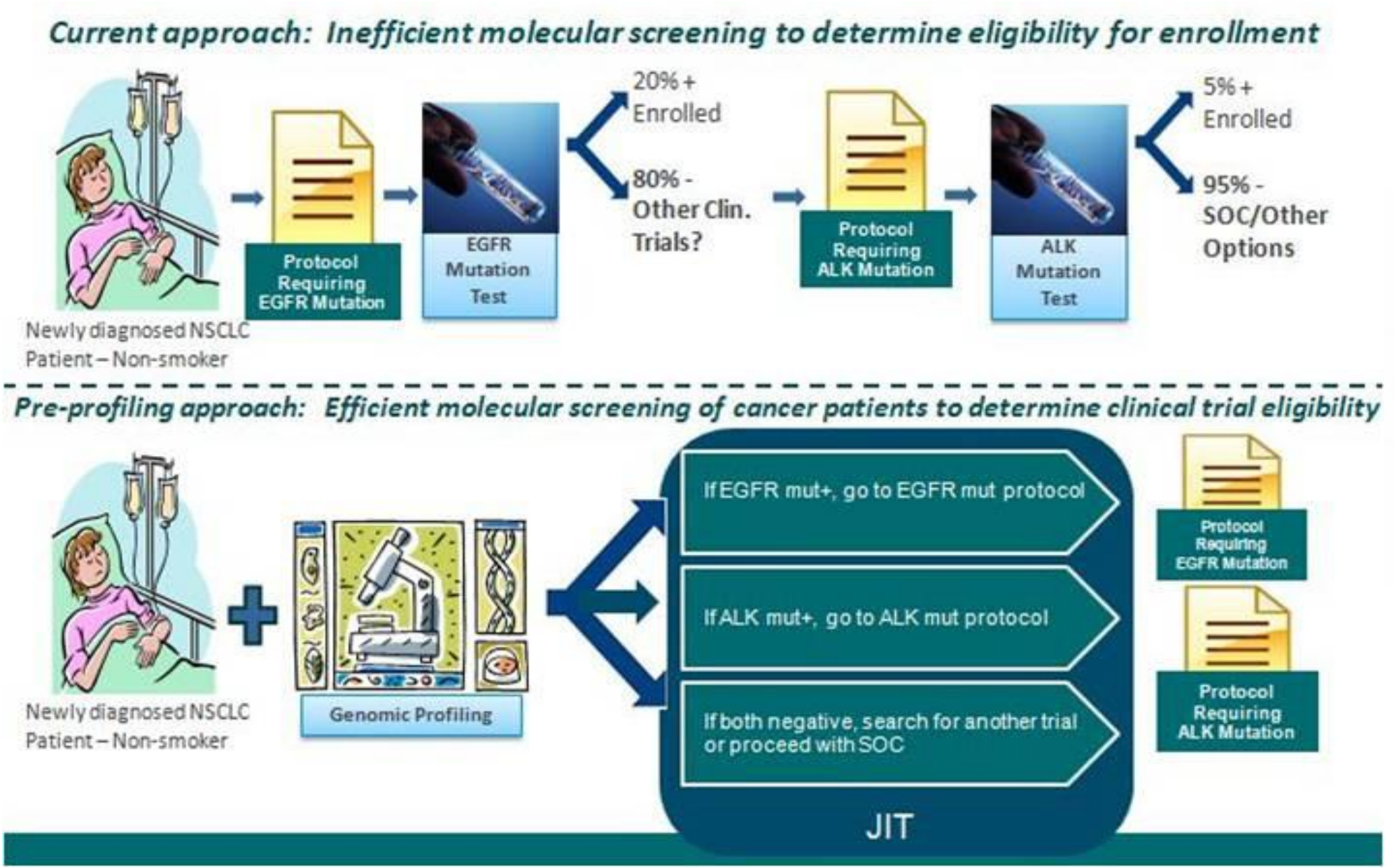
Source: Tufts Center for the Study of Drug Development

COST OF TESTING 2001-2015





Source: NIH



Source:quintiles.com

TELEMEDICINE STUDY OF POST-ACUTE SEQUELAE OF MILD TO MODERATE COVID-19 IN LITHUANIA

- Observational prospective biomedical study
- Performed in Lithuania at 10 study sites in 2021-2022.
- 180 participants with a recent (up to 30 days) COVID-19 diagnosis were enrolled.
- E-technologies that were available via secure application on participant‘s mobile device:
- eConsent
- TeleMedicine platform (allowing video conference between participant and Investigator)
- Electronic Patient Reported Outcomes (ePROs)Results for 166 participants who have completed Subject Satisfaction questionnaires for satisfaction with virtual visits and telemedicine technology

Satisfaction with virtual visits:

Very satisfied – 52 (31,3%)
Satisfied – 55 (33.1%)
Neutral – 28 (16.9%)
Unsatisfied – 2 (1.2 %)
Very unsatisfied – 0 (0%)

Satisfaction with telemedicine technology:

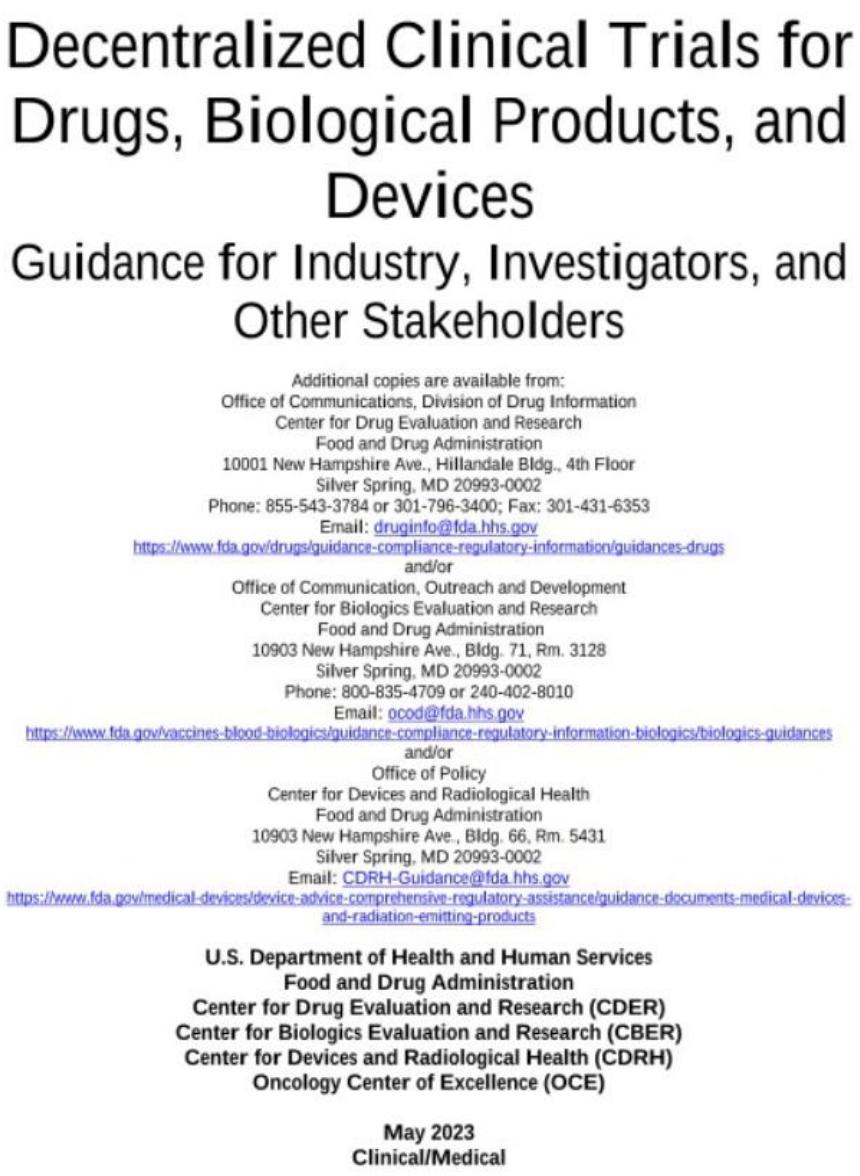
Very satisfied – 53 (31.9%)
Satisfied – 62 (37.3%)
Neutral – 19 (11.4%)
Unsatisfied – 3 (1.8)
Very unsatisfied – 0 (0%)

Source: Biomapas UAB

Decentralized Clinical Trials for Drugs, Biological Products, and Devices

Guidance for Industry, Investigators, and Other Stakeholders
DRAFT GUIDANCE FDA

May 2023



Decentralized Clinical Trials for Drugs, Biological Products, and Devices

Guidance for Industry, Investigators, and Other Stakeholders

Additional copies are available from:
Office of Communications, Division of Drug Information
Center for Drug Evaluation and Research
Food and Drug Administration
10001 New Hampshire Ave., Hillandale Bldg., 4th Floor
Silver Spring, MD 20993-0002
Phone: 855-543-3784 or 301-796-3400; Fax: 301-431-6353
Email: druginfo@fda.hhs.gov
https://www.fda.gov/drugs/guidance-compliance-regulatory-information/guidances-drugs
and/or
Office of Communication, Outreach and Development
Center for Biologics Evaluation and Research
Food and Drug Administration
10903 New Hampshire Ave., Bldg. 71, Rm. 3128
Silver Spring, MD 20993-0002
Phone: 800-835-4709 or 240-402-8010
Email: ocod@fda.hhs.gov
https://www.fda.gov/vaccines-blood-biologics/guidance-compliance-regulatory-information-biologics/biologics-guidances
and/or
Office of Policy
Center for Devices and Radiological Health
Food and Drug Administration
10903 New Hampshire Ave., Bldg. 66, Rm. 5431
Silver Spring, MD 20993-0002
Email: CDRH-Guidance@fda.hhs.gov
https://www.fda.gov/medical-devices/device-advice-comprehensive-regulatory-assistance/guidance-documents-medical-devices-and-radiation-emitting-products

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Center for Devices and Radiological Health (CDRH)
Oncology Center of Excellence (OCE)

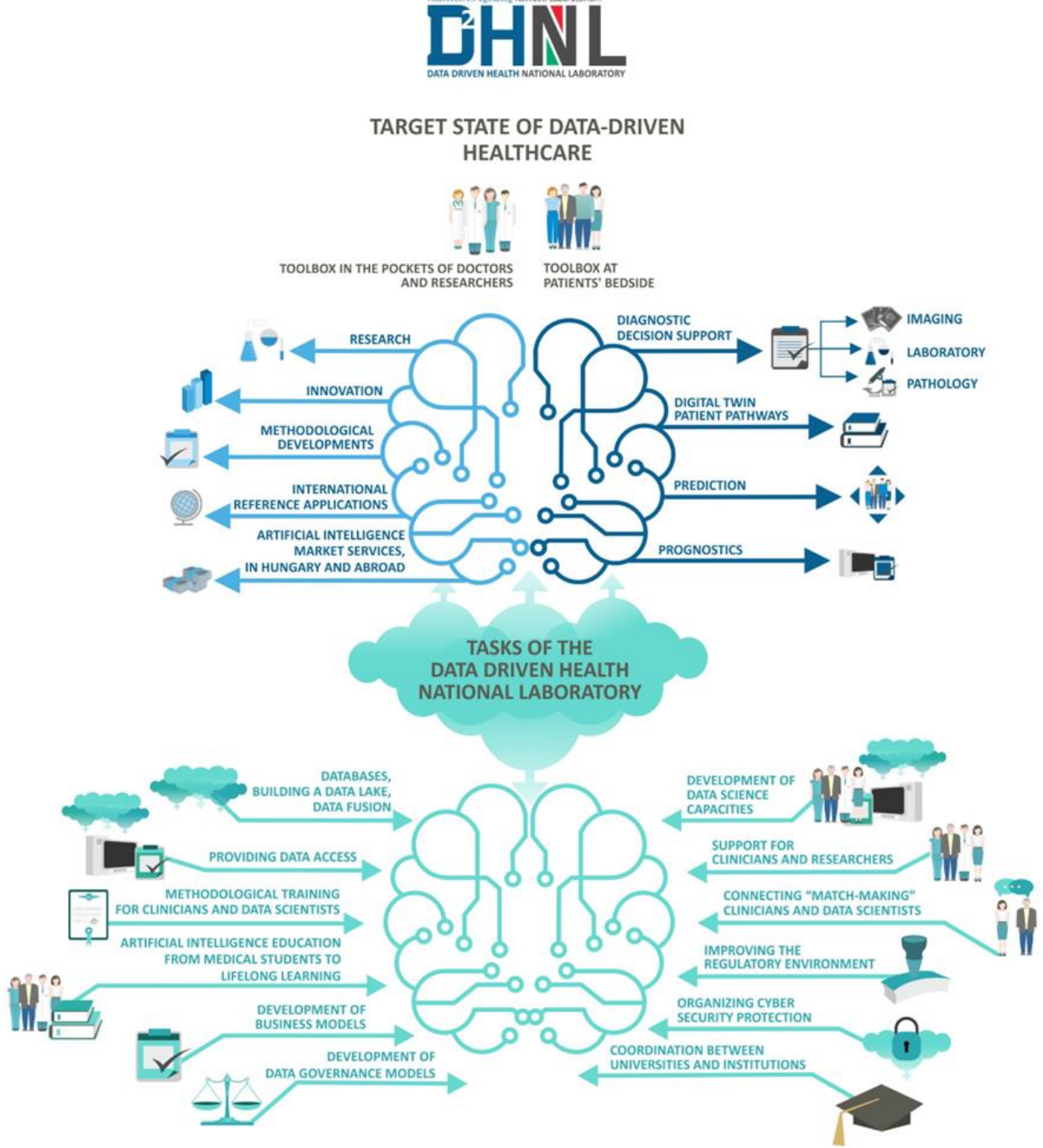
May 2023
Clinical/Medical

Proposal for a Regulation on the European Health

European Health Data Space(EHDS)

- It sets out rules, common standards, infrastructures and a governance framework for the use of electronic health data for healthcare, research, innovation and policy making
- Empower individuals to access and control their personal health data
- Unleash the data economy by fostering a genuine single market for digital health services and products (EHR systems)
- Ensure a consistent framework for the use of individuals' health data for research, innovation, policy-making and regulatory activities

European Commission



Source: Institute of Digital Health, Semmelweis University

E-HEALTH

	Claims $	Clinical trials	Clinical setting Hospital	Pharmacy	Patient
Types of Data	Medical claims	Clinical trial data	HER data	Point of sale data	Patient reported outcomes
			Genomic data		
			Lab tests		Social media
	Prescription drug claims		Specimen/tissue pathology data	Prescription fill data	“Patient utility data”
Primary aggregators/users	State Medicaid	NIH	Providers	Pharmacies	Patients
	Insurance companies	FDA	Clinical labs		
	CMS	Pharma and device companies	Private genetic test companies	Prescription benefit managers	Patient communities/social networks
Secondary aggregators/users	Insurance company analytics subsidiaries	Third party analytics	Genomic databases	Private sector pharmacy data aggregators	Patient advocacy organizations
	FDA sentinel	Patient advocacy organizations	Professional society clinical registries		Patient powered networks
	All payers claims databases		PCORnet		

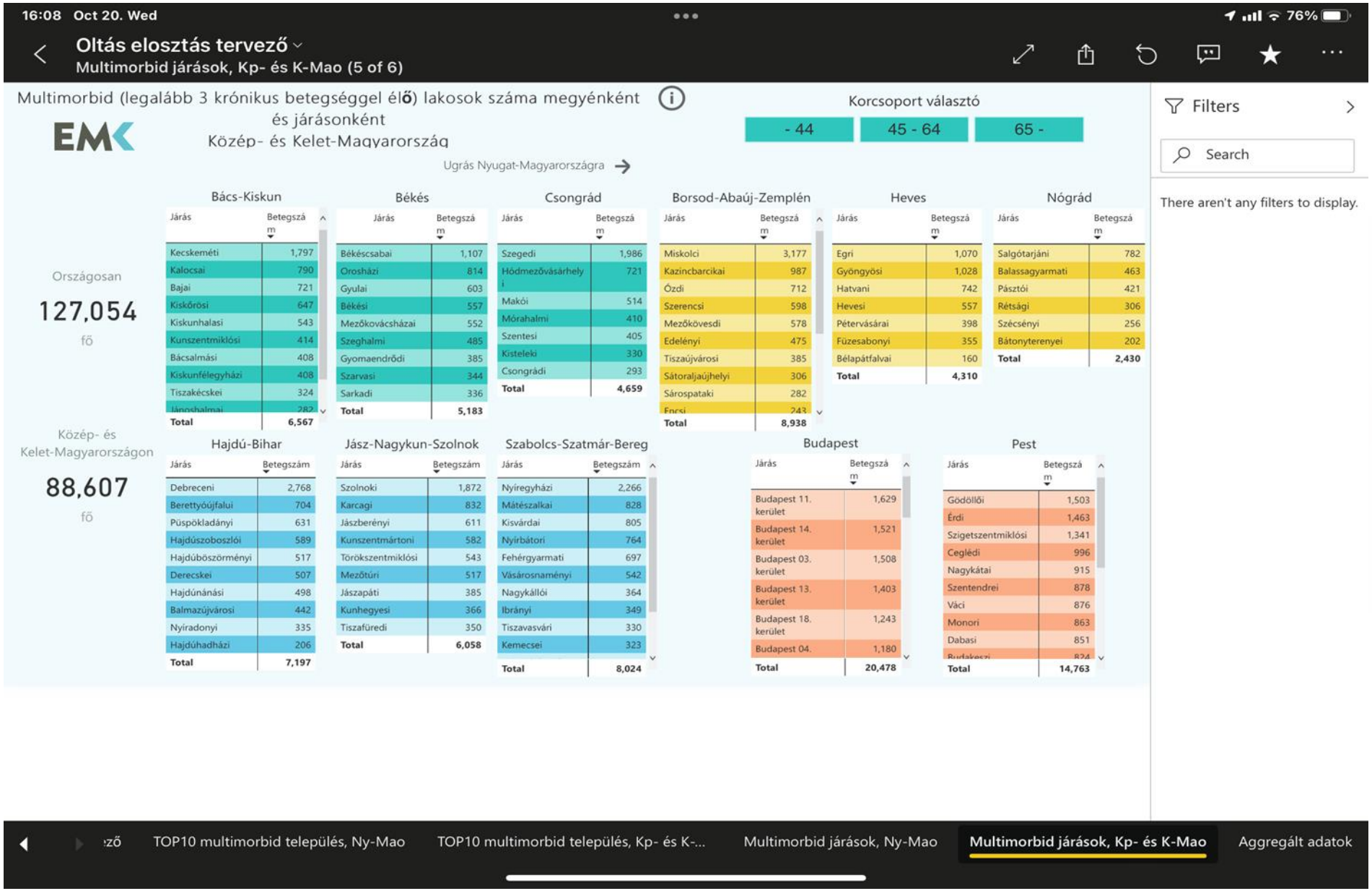
Patient` s data:

- Demographic
- Laboratory
- Radiology
- Diagnosis
- Notes
- Problems
- Insurance
- Activity
- Pathology
- Physicians visited

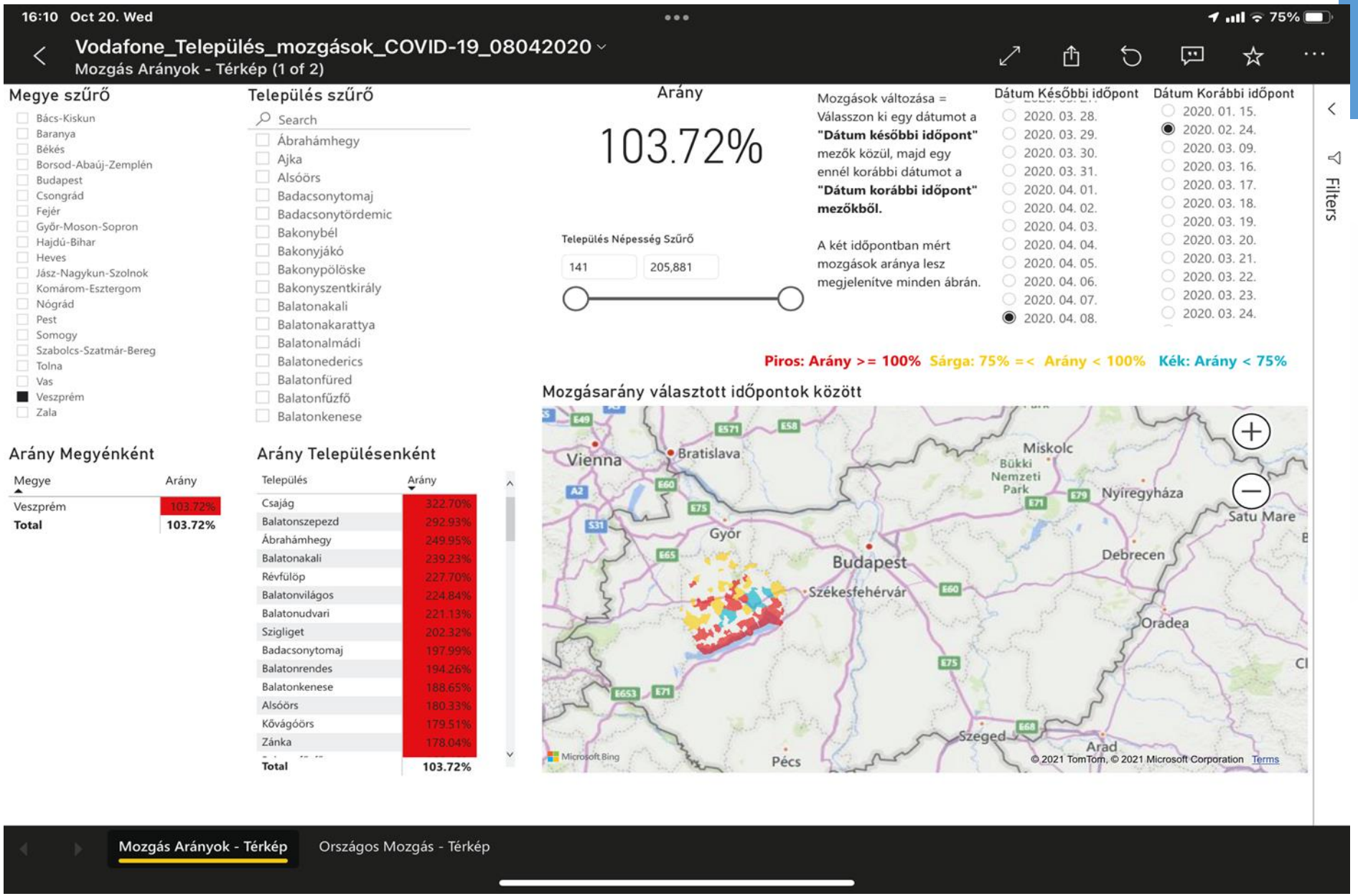
Physician`s data:

- Treatment standards
- Patient visit frequency
- Diagnostic tools usage
- Procedures frequency /cost
- Drug prescription

Modified based on Source: quintiles.com



Source: Institute of Digital Health, Semmelweis University



Source: Institute of Digital Health, Semmelweis University

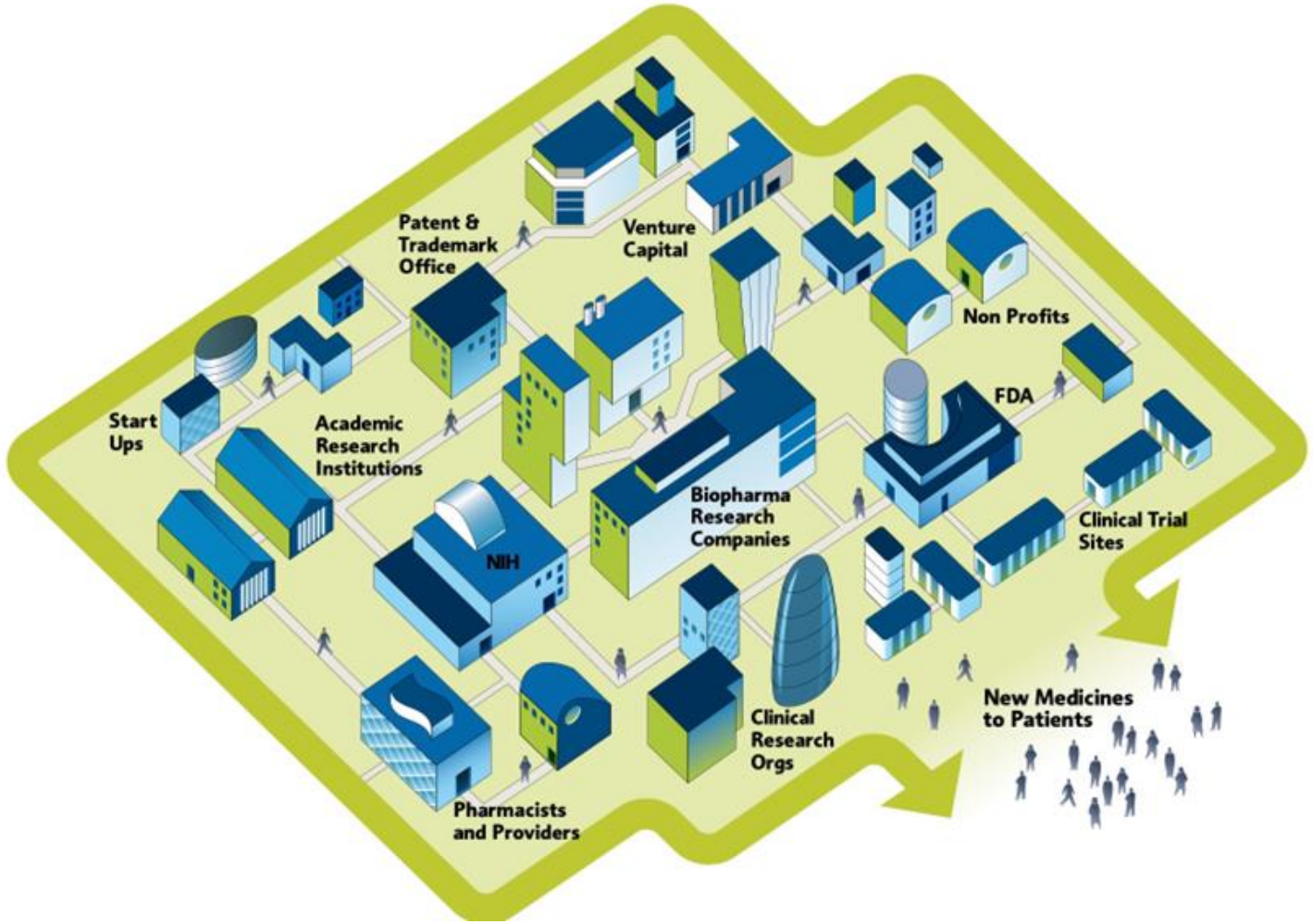
HIGH-LEVEL DIRECTIONS

- Growing global demand for clinical trials and the need for diverse research sites

- Advantages of Small Countries in Clinical Research:
  Cost-effectiveness and favourable regulatory environments
  Streamlined Processes: faster study start-up times and efficient regulatory & patient pathways, Acceptance of new innovative approaches
  Homogeneous populations: genetic or epidemiological studies
  Collaborative networks and partnerships with larger research hubs in Europe, Baltic Clinical Research Network
  Research Infrastructure and Expertise, Investments in state-of-the-art research infrastructure, including clinical trial centres, hospitals, and research institutions Therapeutic area focus

- Funding and Incentives: funding opportunities and incentives for clinical research in small countries, such as grants, tax benefits, or research funding programs

Biopharmaceutical R&D Ecosystem: Delivering New Medicines to Patients



Let's power the journey. Thank you.


CREATING THE FUTURE OF MEDICINE