



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

Accelerating Growth, Driving Innovation

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Data is the next frontier

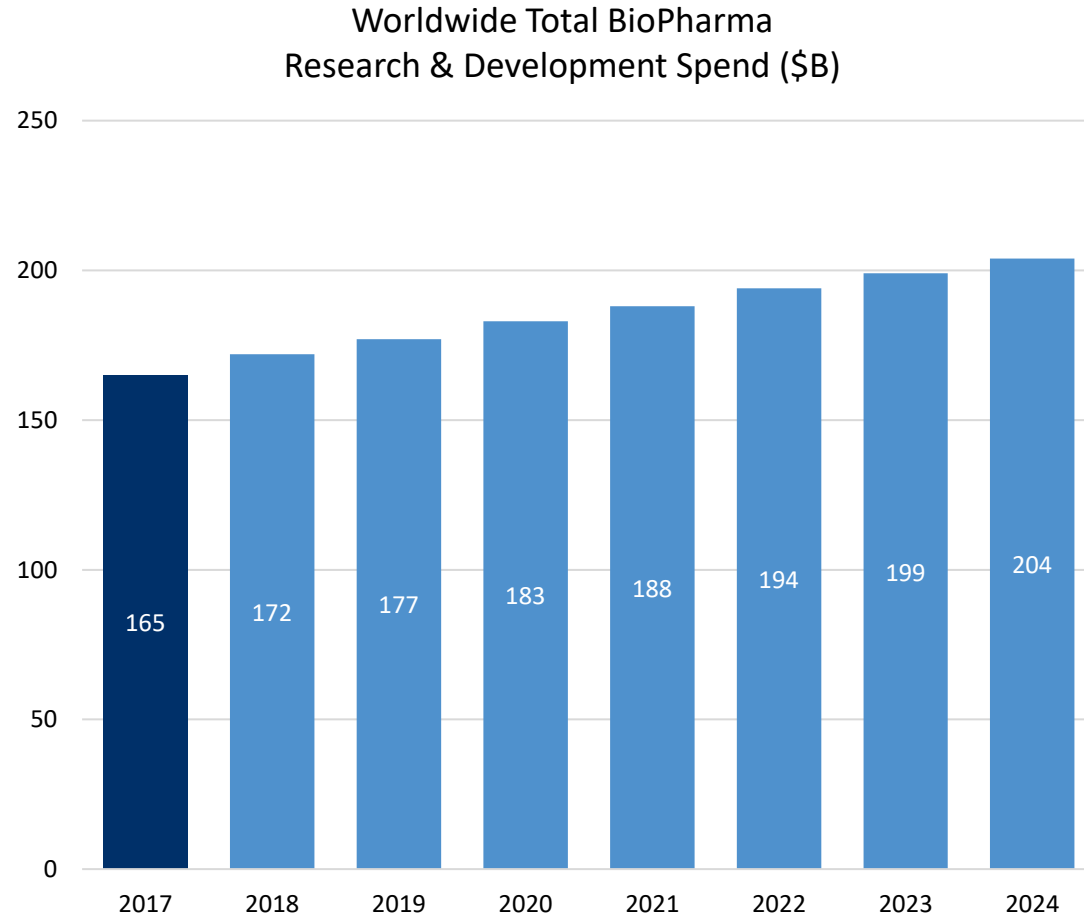


AGENDA

- 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



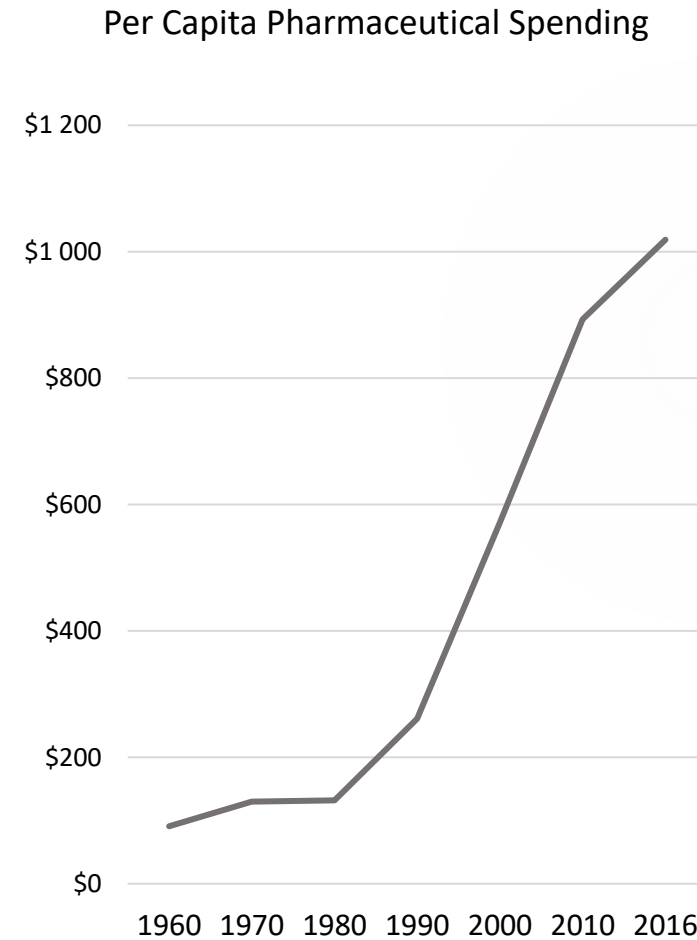
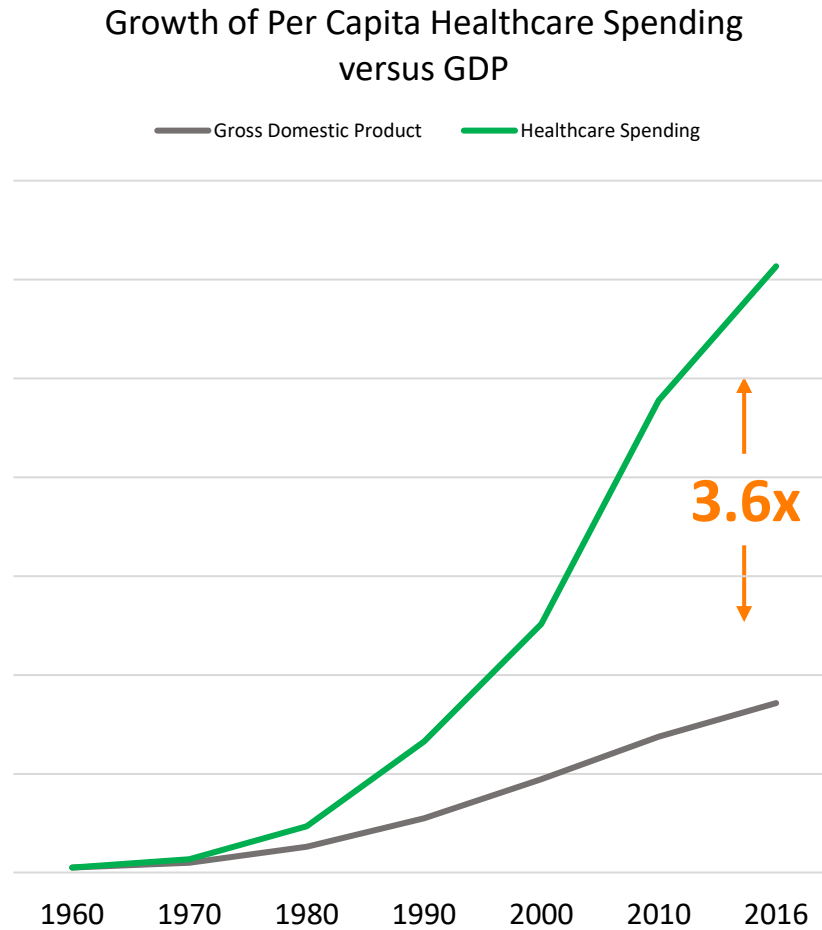
Source: Evaluate Pharma, June 2018; Catalent, Statista; Pharmaprojects 2017

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

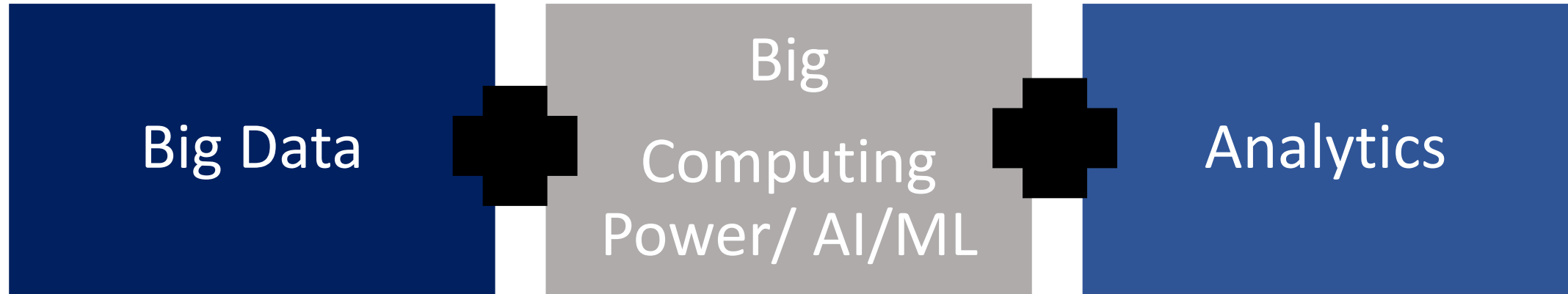
MARKET DRIVERS

The biopharmaceutical industry is driven by waves of spending



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

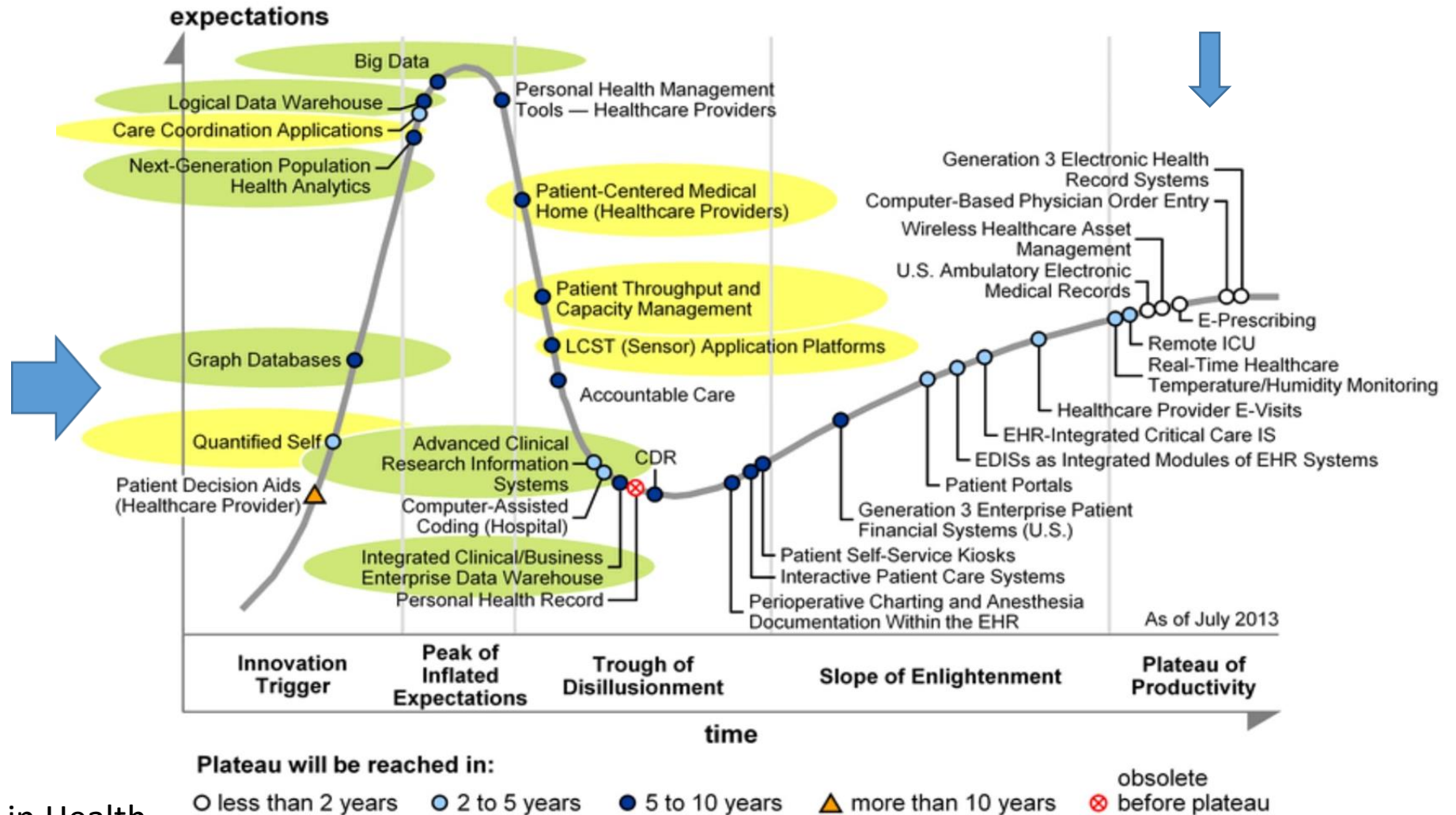
PARADIGM SHIFT



THE NEW CLINICAL TRIAL

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

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



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

Implications for stakeholders



Accelerating Clinical Development

CHALLENGE AND OPPORTUNITIES

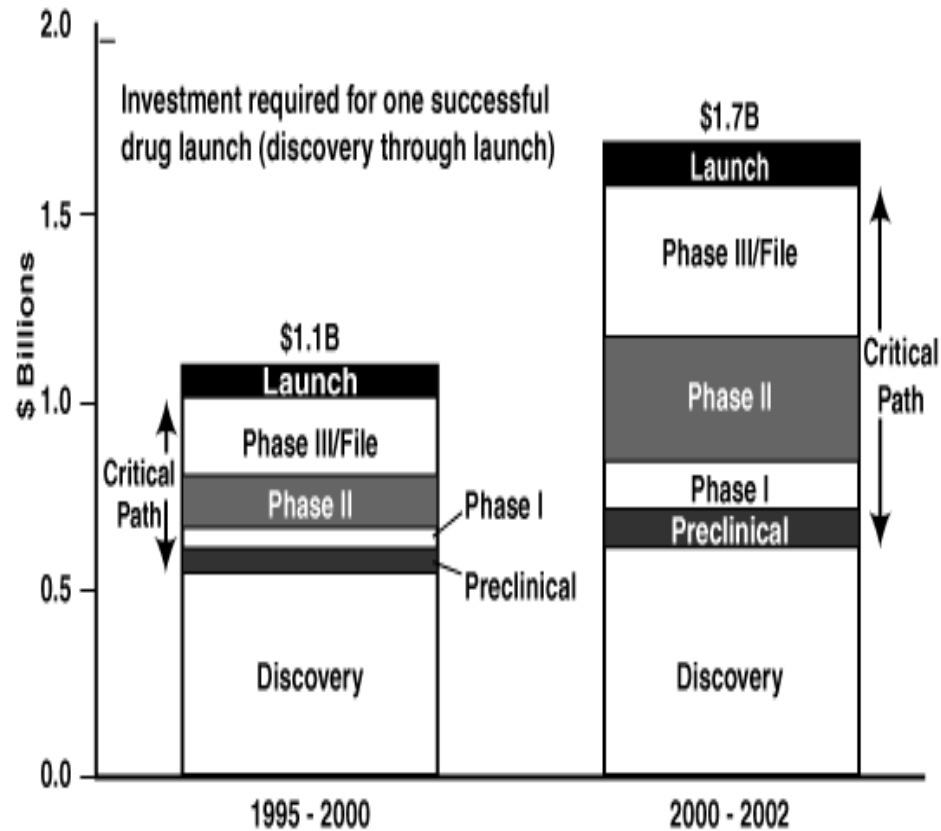
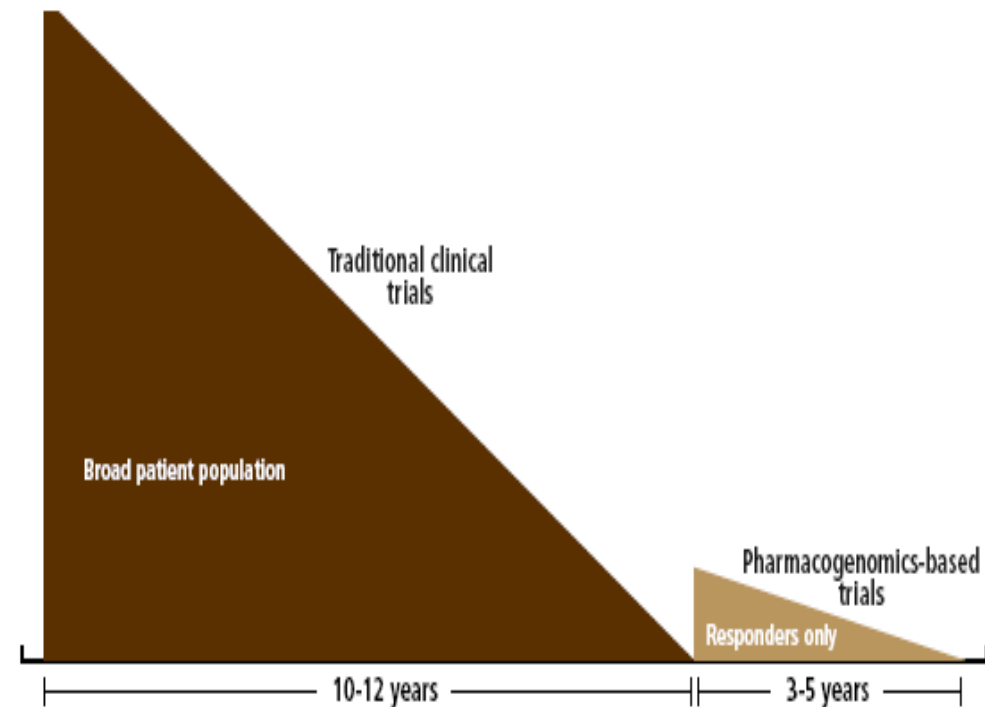


Figure 1: HOW PHARMACOGENOMICS CAN STREAMLINE CLINICAL TRIALS



Source: Quintiles Transnational, 2004

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.

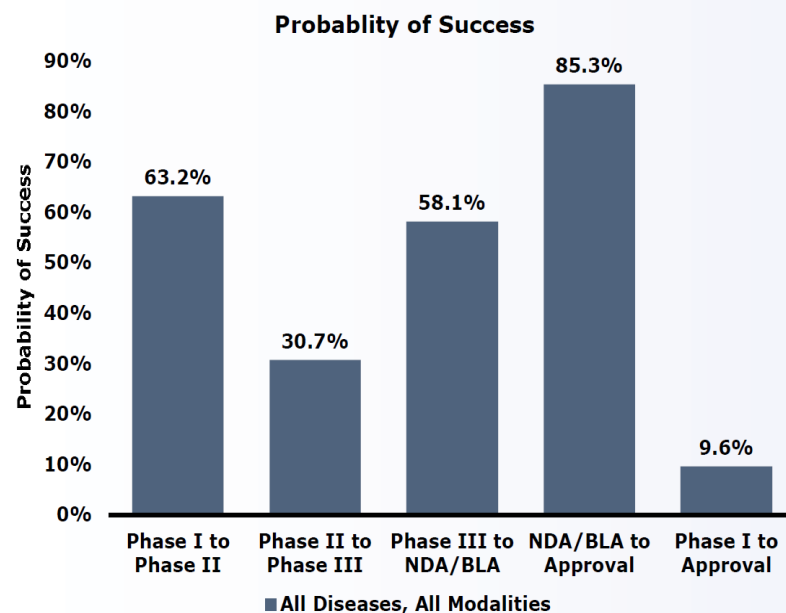
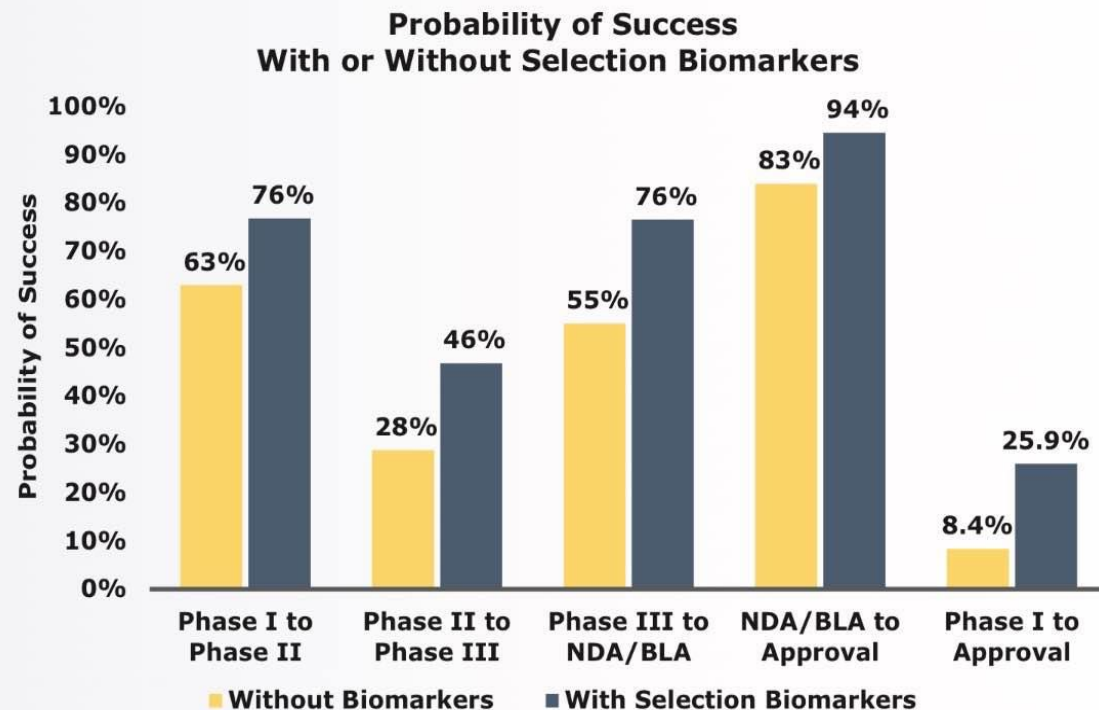


Figure 1. Phase transition success rates and LOA from Phase I for all diseases, all modalities.

- Phase success rate from Phase 1. to LOA (Likelihood 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 On 512 phase transitions out of 9985 (5%) incorporated biomarkers.Phase success rate from Phase 1. to LOA (Likelihood 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

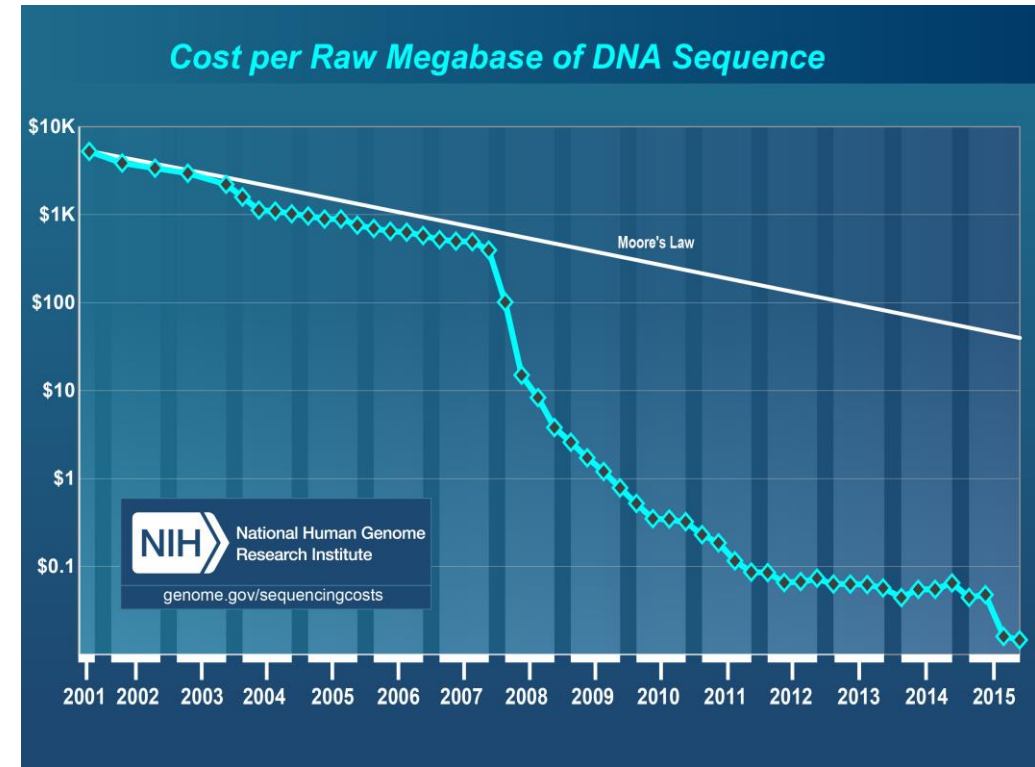
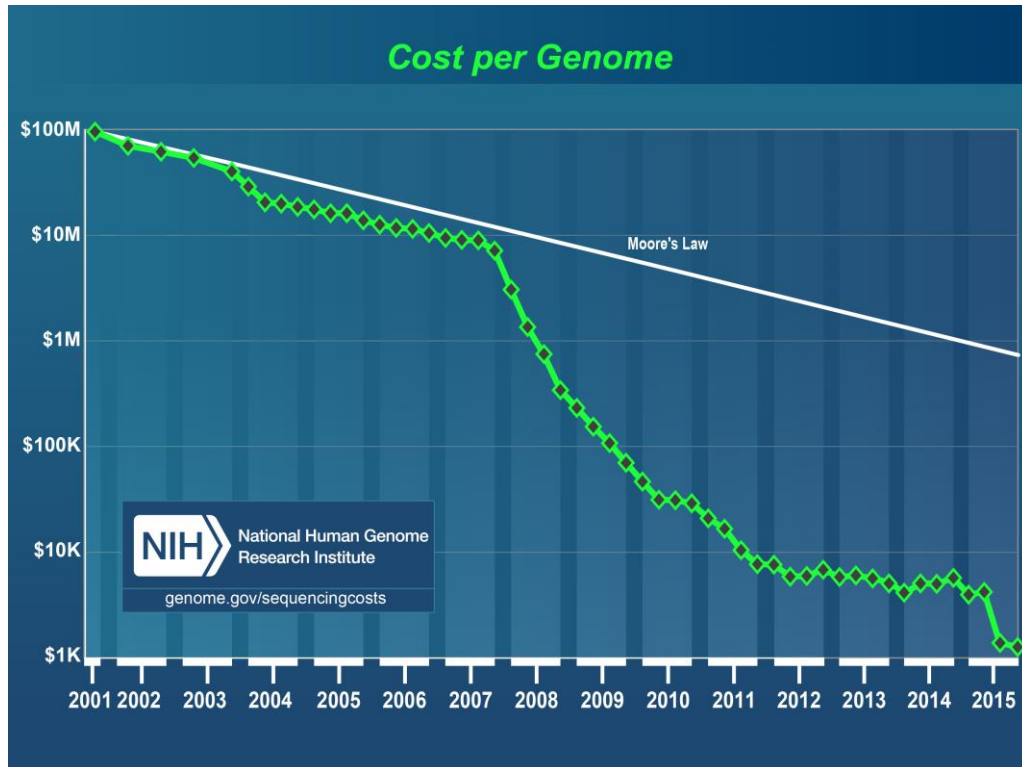
	Cost of CT (% standard költség-beteg)	Subjects No.	Development Time From PH I. To NDA Submission
Xalkori*	100%	960	1.8**
Iressa*	146%	2850	7.0
Tarceva*	154%	3110	5.3

*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

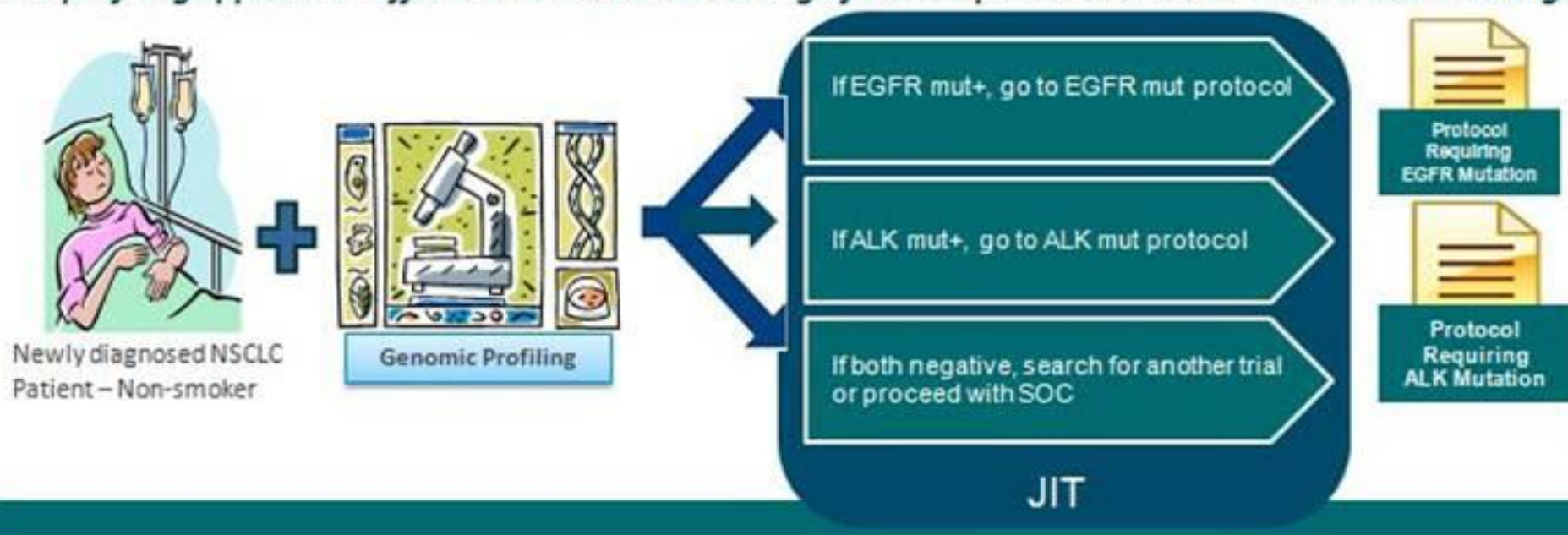


Source: NIH

Current approach: Inefficient molecular screening to determine eligibility for enrollment



Pre-profiling approach: Efficient molecular screening of cancer patients to determine clinical trial eligibility



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%)

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**

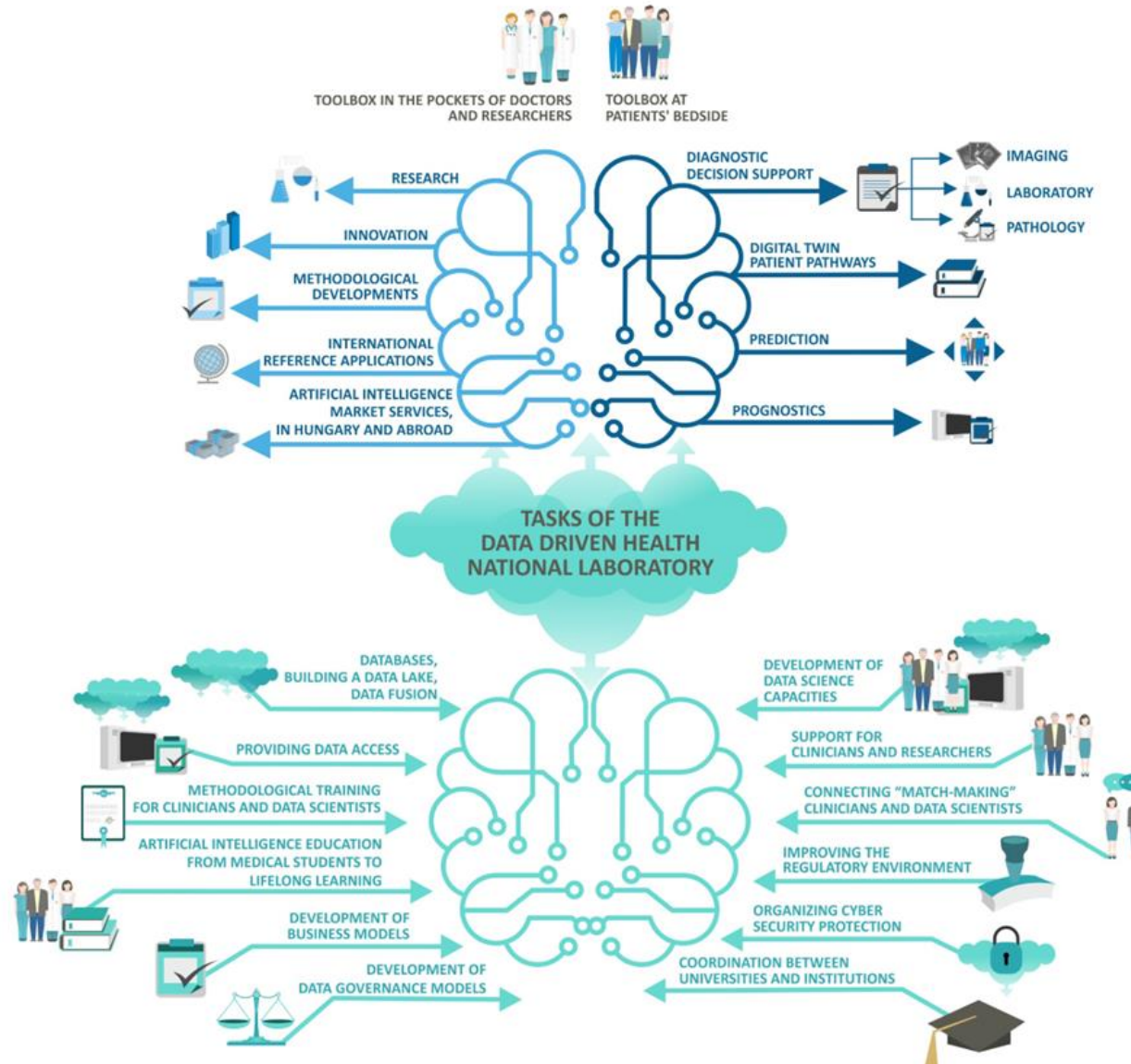
Proposal for a Regulation on the European Health

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



TARGET STATE OF DATA-DRIVEN HEALTHCARE



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



Oltás elosztás tervező

Multimorbid járások, Kp- és K-Mao (5 of 6)



Multimorbid (legalább 3 krónikus betegséggel élő) lakosok száma megyénként



Korcsoport választó

- 44

45 - 64

65 -

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és járásonként
Közép- és Kelet-Magyarország

Ugrás Nyugat-Magyarországra →

Országosan

127,054

fő

Közép- és
Kelet-Magyarországon

88,607

fő

Bács-Kiskun

Járás	Betegszá m
Kecskeméti	1,797
Kalocsai	790
Bajai	721
Kiskőrösi	647
Kiskunhalasi	543
Kunszentmiklósi	414
Bácsalmási	408
Kiskunfélegyházi	408
Tiszakécskei	324
Jánoshalmi	282
Total	6,567

Békés

Járás	Betegszá m
Békéscsabai	1,107
Orosházi	814
Gyulai	603
Békési	557
Mezőkovácsháza	552
Szeghalmi	485
Gyomaendrődi	385
Szarvasi	344
Sarkadi	336
Total	5,183

Csongrád

Járás	Betegszá m
Szegedi	1,986
Hódmezővásárhely	721
Makói	514
Mórahalmi	410
Szentesi	405
Kisteleki	330
Csongrádi	293
Total	4,659

Borsod-Abaúj-Zemplén

Járás	Betegszá m
Miskolci	3,177
Kazincbarcikai	987
Ózdi	712
Szerencsi	598
Mezőkövesdi	578
Edelényi	475
Tiszaújvárosi	385
Sátoraljaújhelyi	306
Sárospataki	282
Ercsi	243
Total	8,938

Heves

Járás	Betegszá m
Egri	1,070
Gyöngyösi	1,028
Hatvani	742
Hevesi	557
Pétervársai	398
Füzesabonyi	355
Bélapátfalvai	160
Total	4,310

Nógrád

Járás	Betegszá m
Salgótarjáni	782
Balassagyarmati	463
Pásztói	421
Rétsági	306
Szécsényi	256
Bátonyterenyi	202
Total	2,430

Hajdú-Bihar

Járás	Betegszá m
Debreceni	2,768
Berettyóújfalui	704
Püspökladányi	631
Hajdúszoboszlói	589
Hajdúböszörményi	517
Derecskei	507
Hajdúnánási	498
Balmazújvárosi	442
Nyíradonyi	335
Hajdúhadházi	206
Total	7,197

Jász-Nagykun-Szolnok

Járás	Betegszá m
Szolnoki	1,872
Karcagi	832
Jászberényi	611
Kunszentmártoni	582
Törökszentmiklósi	543
Mezőtúri	517
Jászapáti	385
Kunhegyesi	366
Tiszafüredi	350
Total	6,058

Szabolcs-Szatmár-Bereg

Járás	Betegszá m
Nyíregyházi	2,266
Mátészalkai	828
Kisvárdai	805
Nyírbátori	764
Fehérgyarmati	697
Vásárosnaményi	542
Nagykállói	364
Ibrányi	349
Tiszavasvári	330
Kemecsei	323
Total	8,024

Budapest

Járás	Betegszá m
Budapest 11. kerület	1,629
Budapest 14. kerület	1,521
Budapest 03. kerület	1,508
Budapest 13. kerület	1,403
Budapest 18. kerület	1,243
Budapest 04. kerület	1,180
Total	20,478

Pest

Járás	Betegszá m
Godóllói	1,503
Érdi	1,463
Szigetszentmiklósi	1,341
Ceglédi	996
Nagykátai	915
Szentendrei	878
Váci	876
Monori	863
Dabasi	851
Rudakeői	824
Total	14,763



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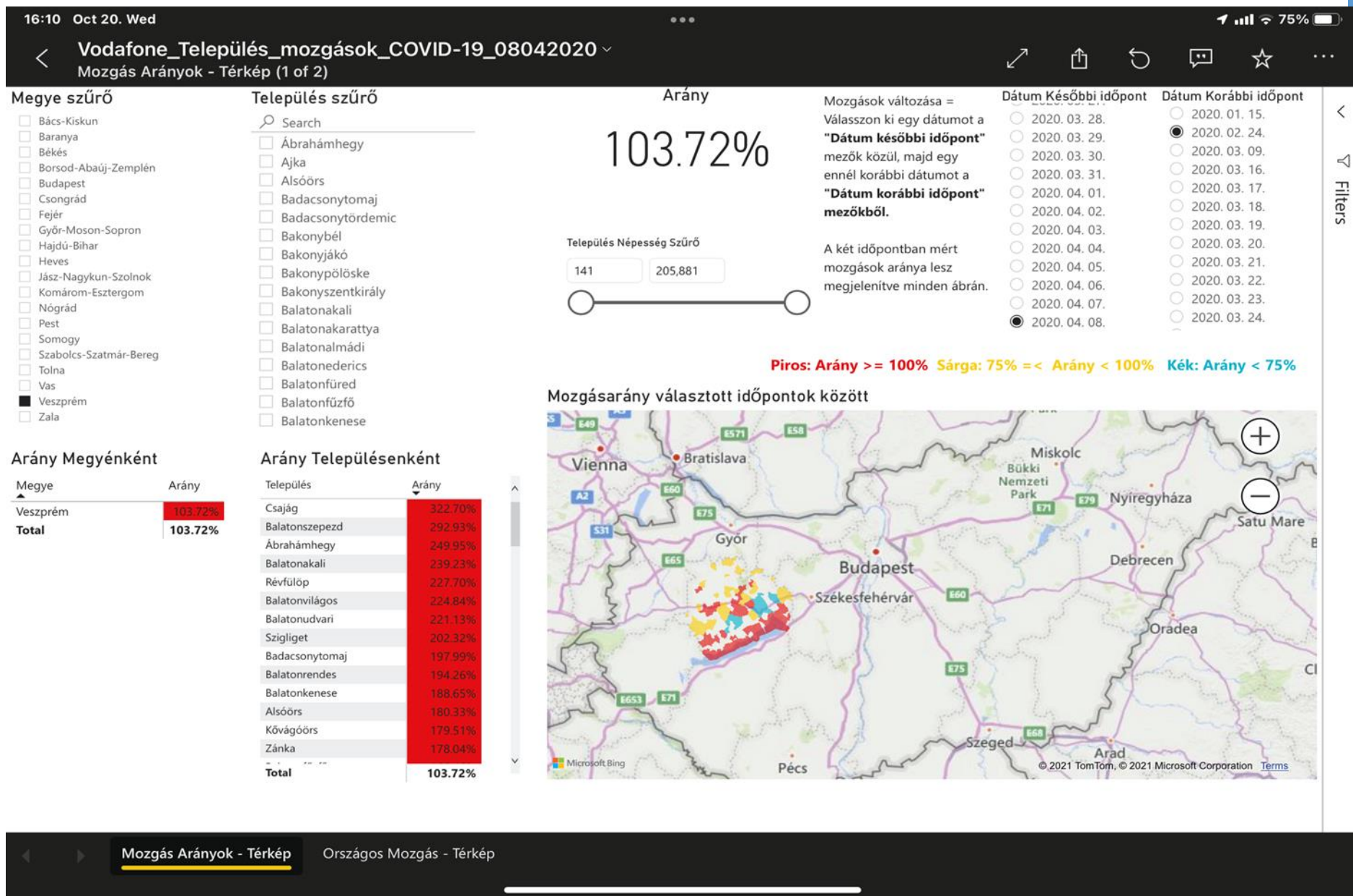
TOP10 multimorbid település, Ny-Mao

TOP10 multimorbid település, Kp- és K-Mao

Multimorbid járások, Ny-Mao

Multimorbid járások, Kp- és K-Mao

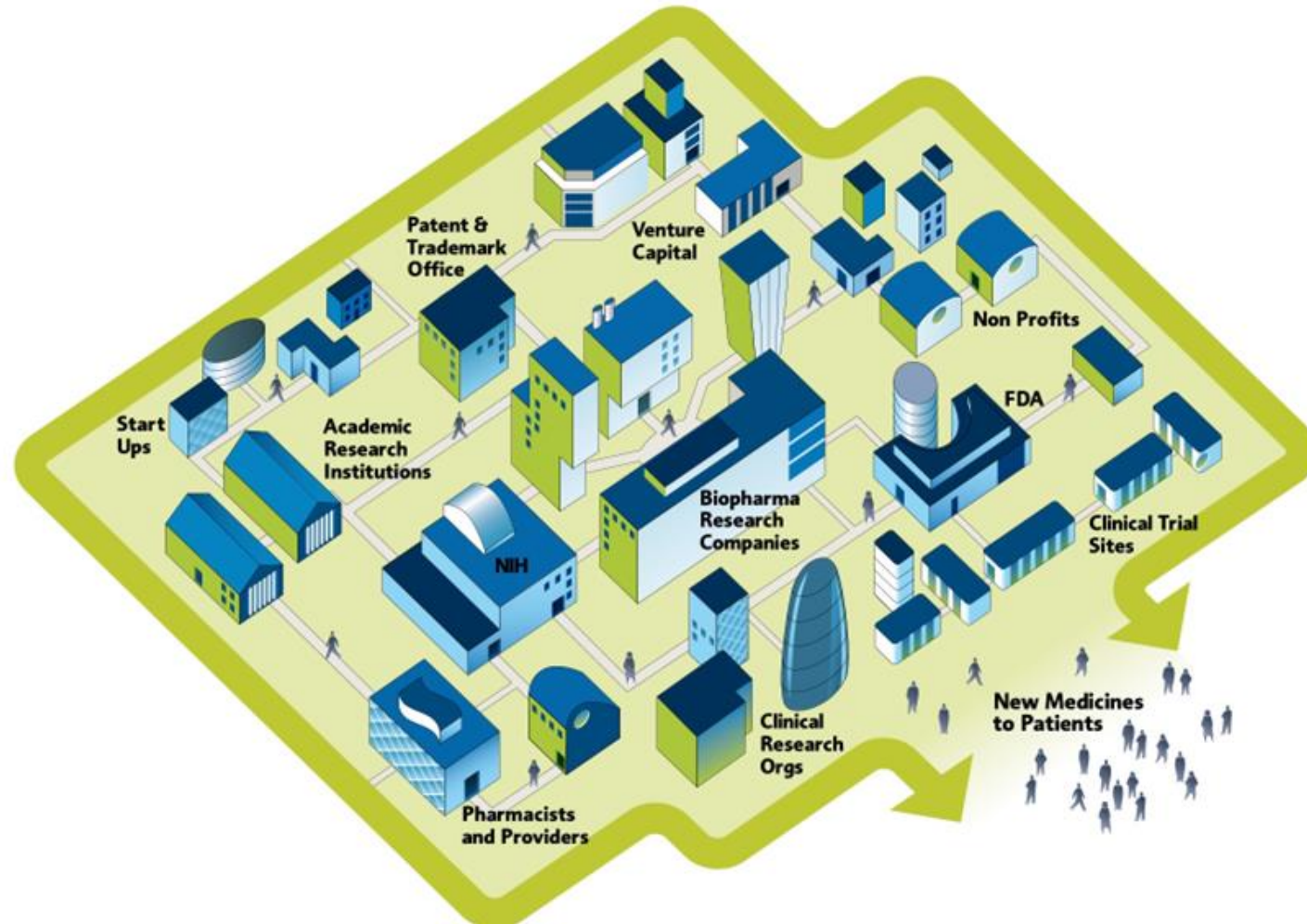
Aggregált adatok



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.

