# Challenging the pharma business model for genomics-based therapeutics

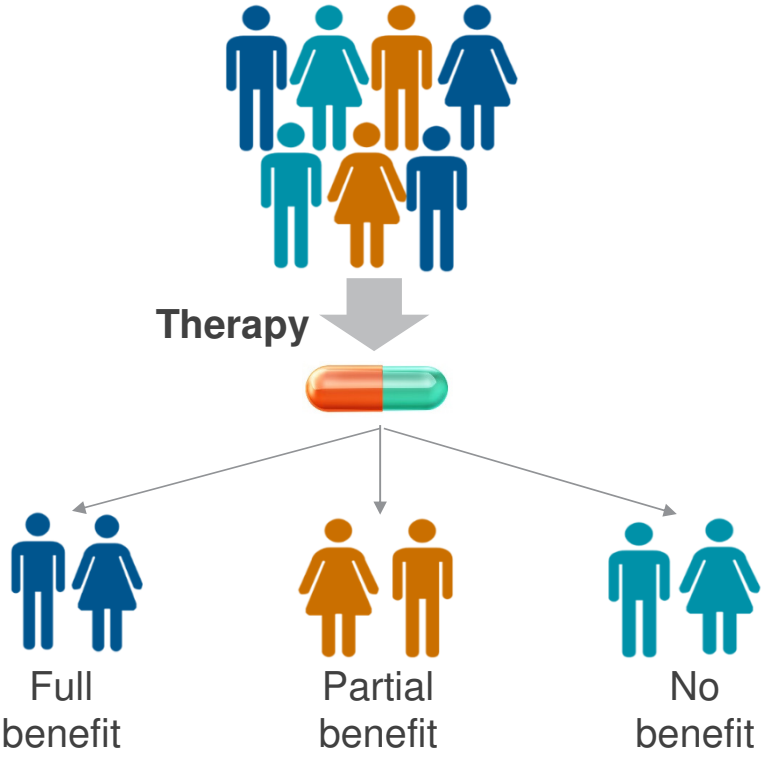
Gregory K. Bell, MBA, PhD
Group Vice President, Global Practice Leader – Life Science

June 24, 2019

CRA Charles River Associates

# Promise of personalized therapeutics

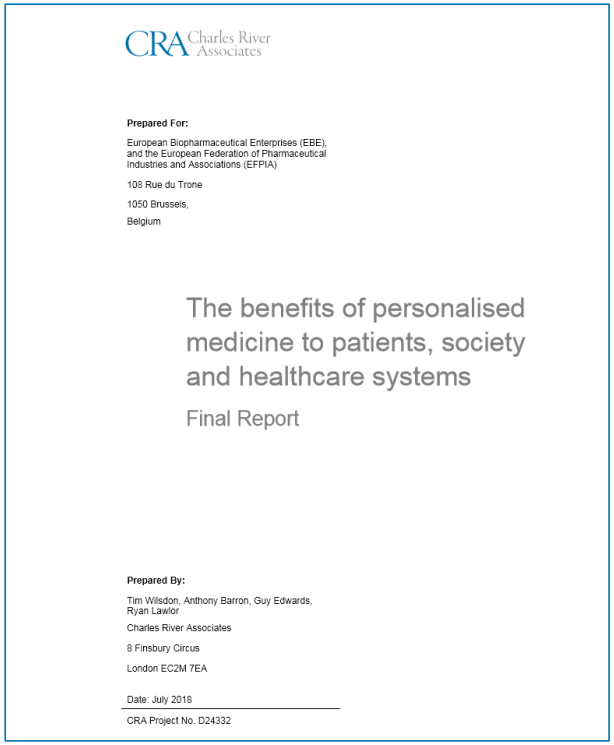
## TODAY: *One-size-fits-all*




## TOMORROW: *Genomics-based*

CRA Charles River Associates

# CRA: Policy implications of personalized medicine



CRA Charles River Associates

**Prepared For:**

European Biopharmaceutical Enterprises (EBE), and the European Federation of Pharmaceutical Industries and Associations (EFPIA)

108 Rue du Trone

1050 Brussels,

Belgium

The benefits of personalised medicine to patients, society and healthcare systems

Final Report

**Prepared By:**

Tim Wilsdon, Anthony Barron, Guy Edwards, Ryan Lawlor

Charles River Associates

8 Finsbury Circus

London EC2M 7EA

Date: July 2018

CRA Project No. D24332

- Objectives:
  - Characterize the benefits of personalized medicine
  - Identify barriers and enablers for development and use of personalized medicine
  - Propose policy recommendations
- Study was completed in July 2018 in collaboration with EBE (European Biopharmaceutical Enterprises) and EFPIA (European Federation of Pharmaceutical Industries and Associations)

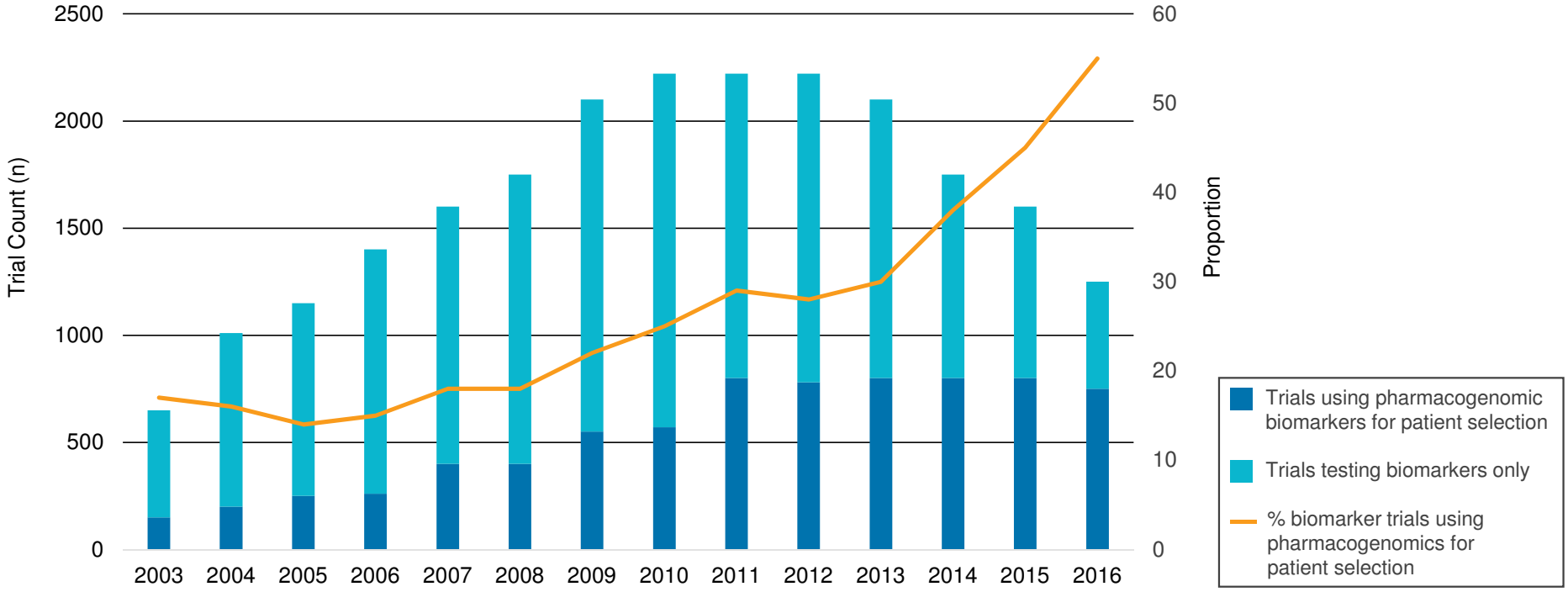
https://www.ebe-biopharma.eu/mediaroom/new-ebe-efpia-study-demonstrates-benefits-of-personalised-medicine-for-patients-society-and-healthcare-systems-and-makes-recommendations-for-equitable-access-for-patients-in-europe/



CRA Charles River Associates

# Genomics are driving clinical trials

## Phase I-IV clinical trials utilizing biomarkers by trial start year, 2003 - 2016






*Source: Pharma Intelligence; The Benefits of Personalised Medicine to patients, society, and healthcare systems, CRA – July 2018.*

CRA Charles River Associates

# Today's focus


*Current pharma business model does not apply for genomics-based personalized therapeutics*

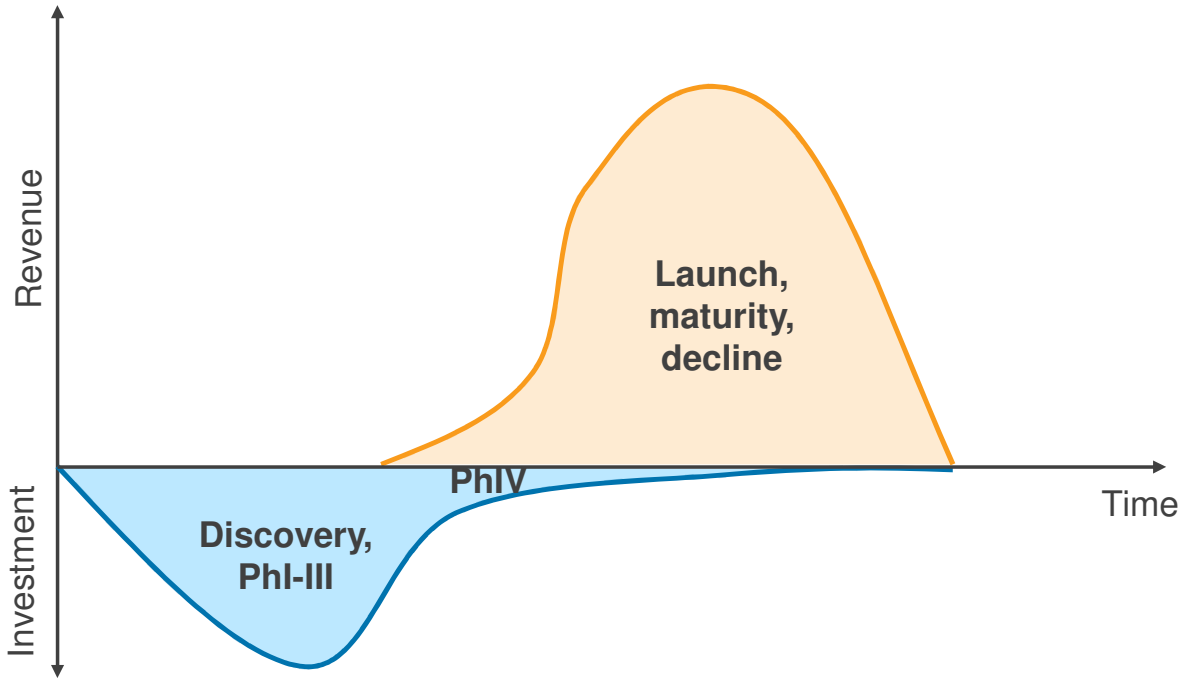

*Value-based models with industry partnerships are needed to support commercialization of personalized therapeutics*



CRA Charles River Associates

# Traditional pharma business model: Recoup R&D costs with 'blockbuster' medicines

## Traditional 'blockbuster' pharma business model



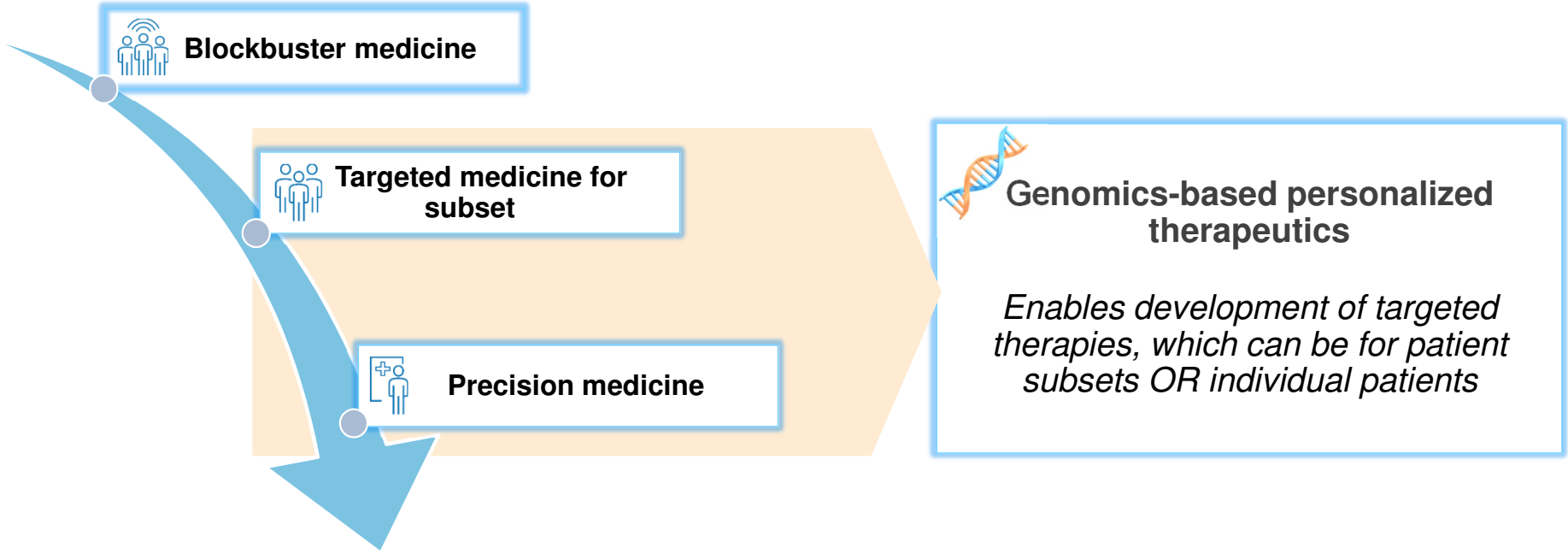

Large number of candidates with significant upfront R&D cost

Small number of drugs with global sales (>$1bn)



CRA Charles River Associates

# Personalized medicine is evolving → Need for commercial innovation in the pharmaceutical industry






CRA Charles River Associates

# With genomics-based personalized therapeutics, the traditional model does not apply

Drug development | Approval & access | Manufacturing & distribution | Sales & marketing

## Blockbuster medicines

- Focus on **mass phenotype**
- Typically, **well-defined clinical trial process**
- **Large investment in clinical trials**
  - Average time from PhI-III of **10 years**
  - Average cost for clinical development and post-approval: **$2.87 bn** per drug*
  - Mean of **800 subjects** per PhIII trial
  - Average success rate of **10%**

## Genomics-based

- **Targeted approach** to drug discovery leveraging genomics databases
- Similar requirement for clinical trials, but possibility of approval on PhII data
  - Possibly **smaller trial sizes** with biomarker-positive participants
- Continued **investment** in data
- Co-development of **companion diagnostic based on biomarkers**
- **Success rate: 25%?**

*2013 dollars

*Source: Changing R&D models in research-based pharmaceutical companies. Schuhmacher A et al, J Transl Med. 2016; 14:105.; Innovation in the pharmaceutical industry: new estimates of R&D costs. DiMasi et al, Journal of health Economics. 2016; 47:20-33.; Cost of Developing Drugs is Insane. Herper, Forbes Oct 2017.*



CRA Charles River Associates

# With genomics-based personalized therapeutics, the traditional model does not apply

Drug development | **Approval & access** | Manufacturing & distribution | Sales & marketing

## Blockbuster medicines

- Regulatory approval based on proof of efficacy and safety
- Price and access reflective of **one-size-fits-all** approach to prescribing
  - Most drugs prescribed today are effective in less than **60%** of treated patients
  - Drugs with less specificity but potentially large volume impact typically face **challenges** in obtaining access as payers are looking for value-for-money

## Genomics-based

- **Value-based** pricing and access
  - **Companion diagnostic** to predict responders included within price / access
- Continued **data collection and management** with statistical analysis to evaluate response to drug in genetic group
- Investment in **additional infrastructure** to support diagnostic testing / product delivery

*Source: Realizing the Promise of Personalized Medicine. Aspinall M and Hamermesh R. Harvard Business Review, 2007.*



CRA Charles River Associates

# With genomics-based personalized therapeutics, the traditional model does not apply

Drug development | Approval & access | **Manufacturing & distribution** | Sales & marketing

## Blockbuster medicines

- **Meet demand**, but critical to success?
- **Global sourcing** to reduce costs
- Leverage **standardized distribution channels**
- **New era of biologics** → increasing value of manufacturing
  - Supply security
  - Yield rates and cost efficiency

## Genomics-based

- **Integral part of care delivery**
- **Supply chain innovation** likely to become critical for success
  - Specialized labour and distribution
- **Supply chain security and coordination**
  - Personal information
- Supply costs become **significant component of therapy cost**

*Source: Perspectives and Learnings in Life Sciences Innovation: Opportunities for Growth. Takizawa, B. March 2018.*



CRA Charles River Associates

# With genomics-based personalized therapeutics, the traditional model does not apply

Drug development | Approval & access | Manufacturing & distribution | **Sales & marketing**

## Blockbuster medicines

- **Large, dispersed salesforce**
  - 100,000 reps in 2005 down to 71,000 reps in 2016 in the U.S. market
  - Typically **primary care** outpatient physician targets (e.g., 100,000+ physicians within statin market)
- Sales reps play role in **drug-specific education and brand choice**

## Genomics-based

- **Patient finding** is a critical success factor
- Niche salesforce requiring **tailored marketing and different capabilities**
- Sales rep becomes an **account manager**, with multi-faceted role:
  - Greater focus on disease education and therapy awareness (MSL)
  - Screening and patient ID support
  - Explaining reimbursement and providing link to service delivery and CoE

*Source: 5 Trends Shaping the Pharma Sales Force. LaMotta, L. Sept 2017.*

# Today's focus

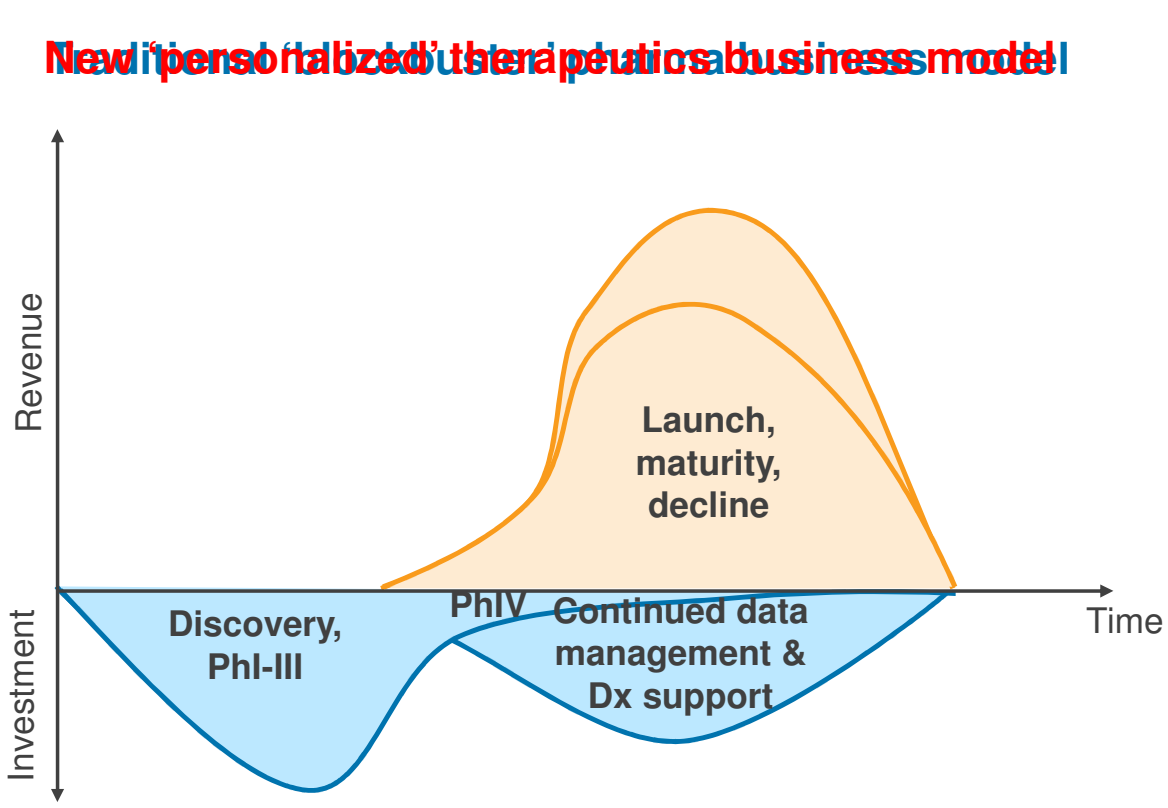

*Current pharma business model does not apply for genomics-based personalized therapeutics*

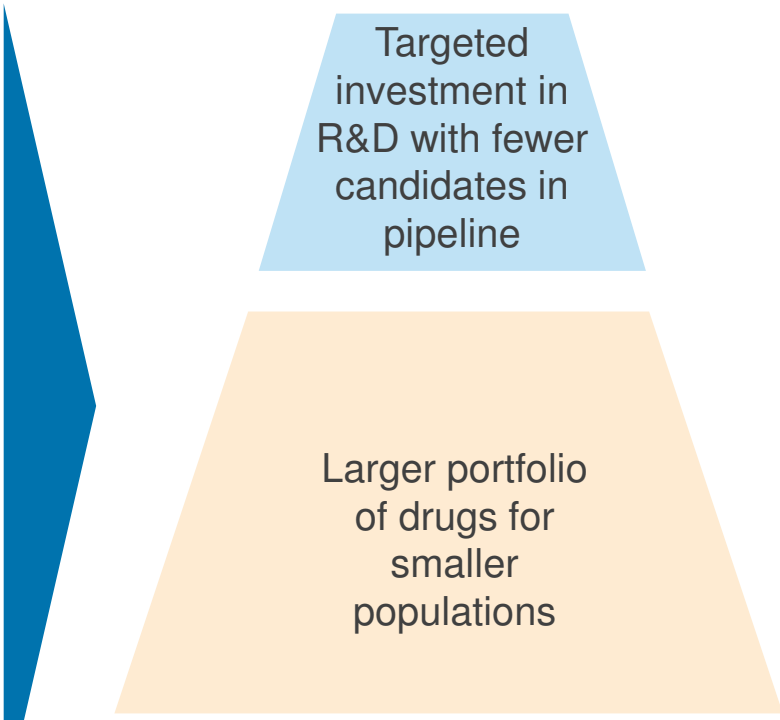

*Value-based models with industry partnerships are needed to support commercialization of personalized therapeutics*



CRA Charles River Associates

# Genomics-based business model requires infrastructure and continued investment to support commercialization





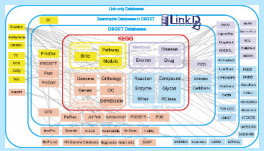

CRA Charles River Associates

# Within drug development, pharma will need partnerships with data companies and infrastructure in regulatory bodies

**Drug discovery & development** | Launch & patient access

*To successfully commercialize a genomics-based personalized Tx, pharma needs…*

*…this requires partnership with…*

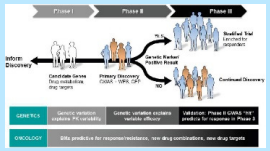


Access to genomic data

23andMe
Helix
ancestry

Personal genomics companies

Country / regional-level population genomics projects



Revised clinical trial design requirements

Health Canada
FDA
PAREXEL

Regulatory / marketing authorization bodies

Contract research organizations for running specialized trials



CRA Charles River Associates

# Strides are being made to develop population-based genomics databases to facilitate drug discovery

| 2018 partnership (not exhaustive) | Details |
|---|---|
| gsk ⟷ 23andMe | • $300 million; DNA chip data, sequenced patients with common diseases |
| Roche ⟷ flatiron | • $1.9 billion; focused on oncology outcomes based on medical data only in the US |
| Roche ⟷ FOUNDATION MEDICINE | • $2.4 billion; >100,000 tumors with panel sequencing of 400 known tumor genes in the US |
| REGENERON, AstraZeneca, abbvie, Pfizer, Biogen, Alnylam ⟷ biobank uk | • 500,000 exomes tied to ICD codes for diseases and diagnostic tests in the UK, targeted for end 2019 |



*Source: Frontline Genomics: The Rise of Population-scale Genomic Projects for Pharma R&D Investments, 25 Apr 2019.*

# To ensure value is realized, countries will need to invest in data infrastructure alongside payer and policy changes

**Drug discovery & development** → **Launch & patient access**

*To successfully commercialize a genomics-based personalized Tx, pharma needs…*

*…this requires partnership with…*

Value realization through pricing, access, and policy

CADTH pCPA

Payers / HTA organizations responsible for value assessment, pricing & access

Investment in genomics databases linked to health records to capture outcomes

National and international patient registries and genomics databases



CRA Charles River Associates

# Once on the market, pharma companies will require partnerships with providers and diagnostics companies to reach their patients

**Drug discovery & development** → **Launch & patient access**

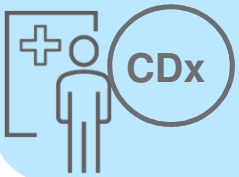
*To successfully commercialize a genomics-based personalized Tx, pharma needs…*

Patient finding support (screening tools, access to individual patients)

*…this requires partnership with…*

23andMe — Genomic data

UHN Princess Margaret Cancer Centre — Healthcare providers / CoEs




Companion diagnostics for patient treatment selection and testing infrastructure

Roche Diagnostics; Bayer Diagnostics — IVD / molecular diagnostics companies

GEN-PROBE — Laboratories



CRA Charles River Associates

# Implementation of companion diagnostic testing can be a challenge without investment in infrastructure

## Current situation regarding implementation of CDx testing

| | |
|---|---|
| **Medicine approval** | Targeted therapy clinical trials are successful & medicines get approved |
| **Medicine reimbursement** | Medicine reimbursement approved (country by country basis) |
| **Lab adoption** | Demand for CDx testing reaches the labs, doctors wish to start ordering testing to prescribe new medicines |
| **Selection of technology** | Labs evaluate various technology and decide which test they wish to adopt |
| **Capacity building** | Labs/healthcare systems invest in infrastructure and expertise |
| **Quality control** | Labs embed the test in established local quality systems; |
| **Clinical Dx service offered** | CDx testing offered to patients as clinical service |
| **Maintenance of quality** | Labs may seek to join External Quality Control programmes and/or obtain ISO accreditation |

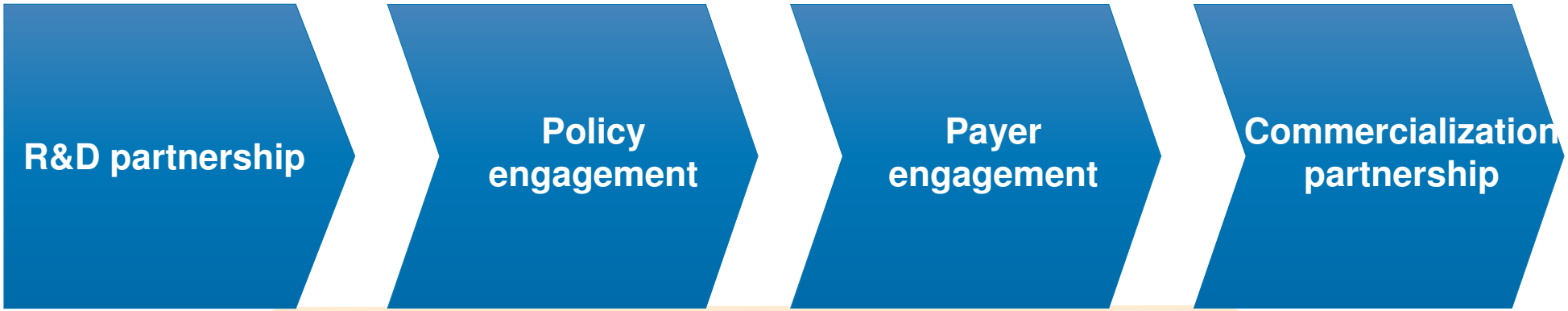


*Source: The Benefits of Personalised Medicine to patients, society, and healthcare systems, CRA – July 2018.*

CRA Charles River Associates

# Industry needs to continue to shape the evolving genomics and personalized medicine environment across markets

## Recommendations for industry






CRA Charles River Associates

**Dr. Gregory K. Bell**

**Group Vice President**

**Global Practice Leader for Life Sciences**

**gbell@crai.com**

**(617) 425-3357**