A Multistakeholder Approach to Enhancing Diversity in Clinical Trials

Maryland Delegate Sheree Sample-Hughes, Eastern Regional Director, Women In Government Board of Directors (Moderator)

Mary Nwokedi, Director of Clinical Trials Diversity, Science & Regulatory Advocacy, PhRMA

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#AccessToHealthCare

Update on Innovation Efforts and Clinical Trial Diversity

Sharon Lamberton, MS, RN, Deputy VP, State Policy

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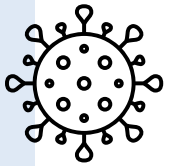
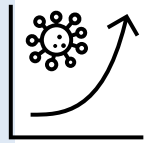
WIG Conference

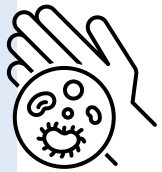
November 17, 2022



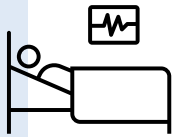
R&D: Risky and Expensive Endeavor



Industry & Government Collaboration



Efforts to Address Clinical Trial Diversity



Assistance/Resources for Patients

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More than 8,000 Medicines in Development Globally[1]

Biopharmaceutical researchers are pursuing many innovative scientific approaches that are driving therapeutic advances.

ALS (Amyotrophic Lateral Sclerosis)

Stem cell therapies aim to replace and/or protect damaged motor neurons and slow disease progression.

ALZHEIMER'S DISEASE

CRISPR-Cas9 genetic screening is being utilized to look for changes in amyloid beta production, a believed cause of the disease. Other approaches include immunotherapies, vaccines, or antibodies that target abnormal tau proteins, which may cause cognitive impairment.

CANCER

Allogeneic, or **"off the shelf," cell therapy** involves the personalized modification of immune-boosting cells, from healthy donors, and infusing them into a cancer patient to target and kill cancer cells. Some of these cell therapies are being designed to be delivered in the outpatient setting.

HEMOPHILIA

Adeno-associated viral (AAV) vector-mediated gene therapies enable patients to clot blood and can reduce the need for chronic treatment to prevent bleeding episodes.

Source: PhRMA analysis of Adis R&D Insight database[1]

New Era of Medicine Transforming Patient Care

50 New Medicines Were Approved by the FDA in 2021, 54% First-In-Class

The Washington Post

Cancer Death Rate

Cancer death rate posts biggest one-year drop ever

- 4 new therapies for non-small cell lung cancer

CBS

Game Changer

Newly approved drug being called ‘game changer’ for people who suffer from hemophilia

REUTERS

Coronavirus Vaccine

Reasons for hope: the drugs, tests and tactics that may conquer coronavirus

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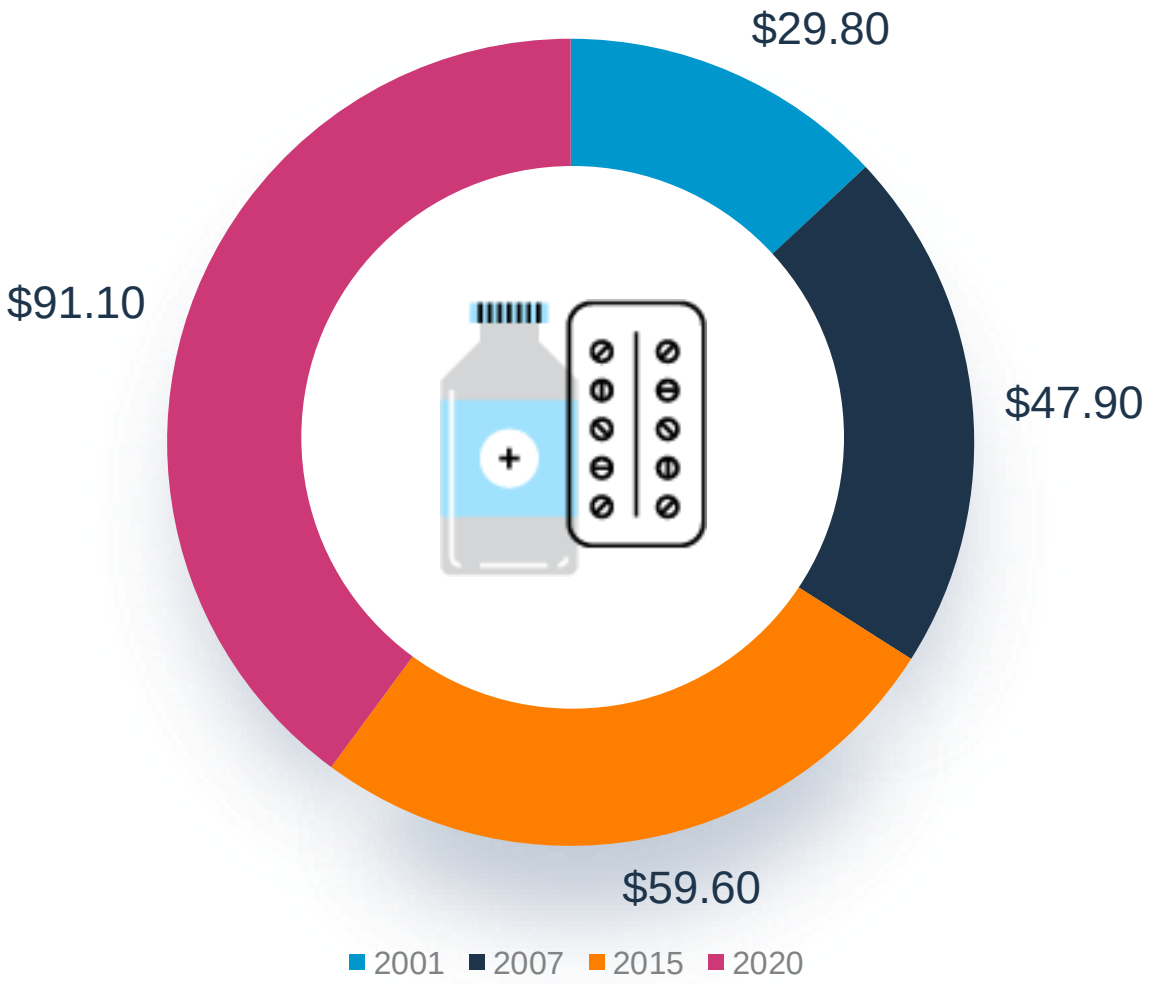
Source: US FDA. Center for Drug Evaluation and Research (CDER) Advancing Health through Innovation. 2020 New Drug Approvals.
Note: Due to lack of data availability, novel approvals are not inclusive of medicines approved by the Center for Biologics Evaluation and Research (CBER) in 2020.

Unprecedented Investment

Record **$102.3 billion** in R&D investment in 2021 alone

Among the **highest R&D intensity ratios** of all industries

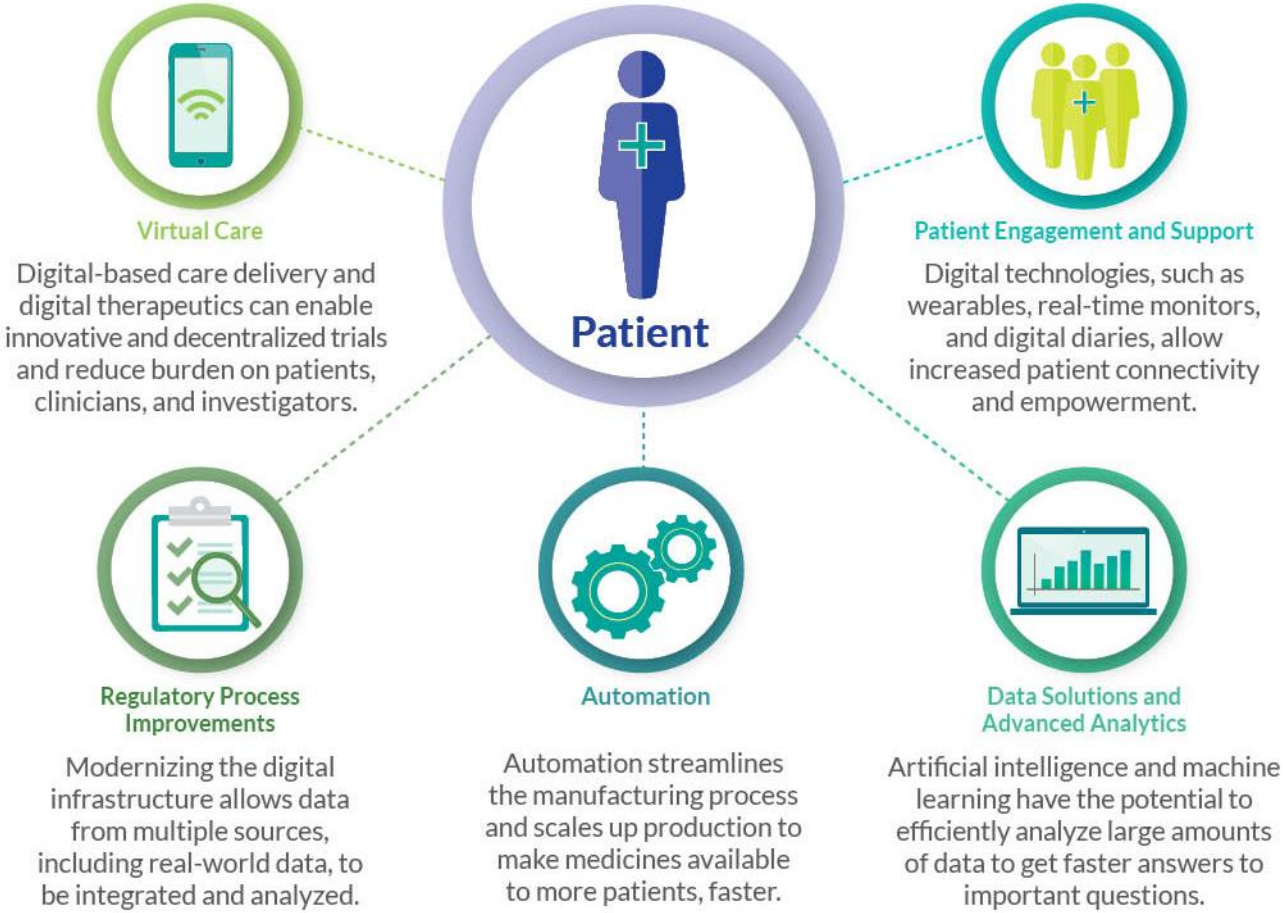


PhRMA Member Company R&D Expenditures
2001-2020 ($Billions)

Source: PhRMA Member Annual Survey, 2021

Digital Technologies May Enhance Drug Development and Improve Patient Care

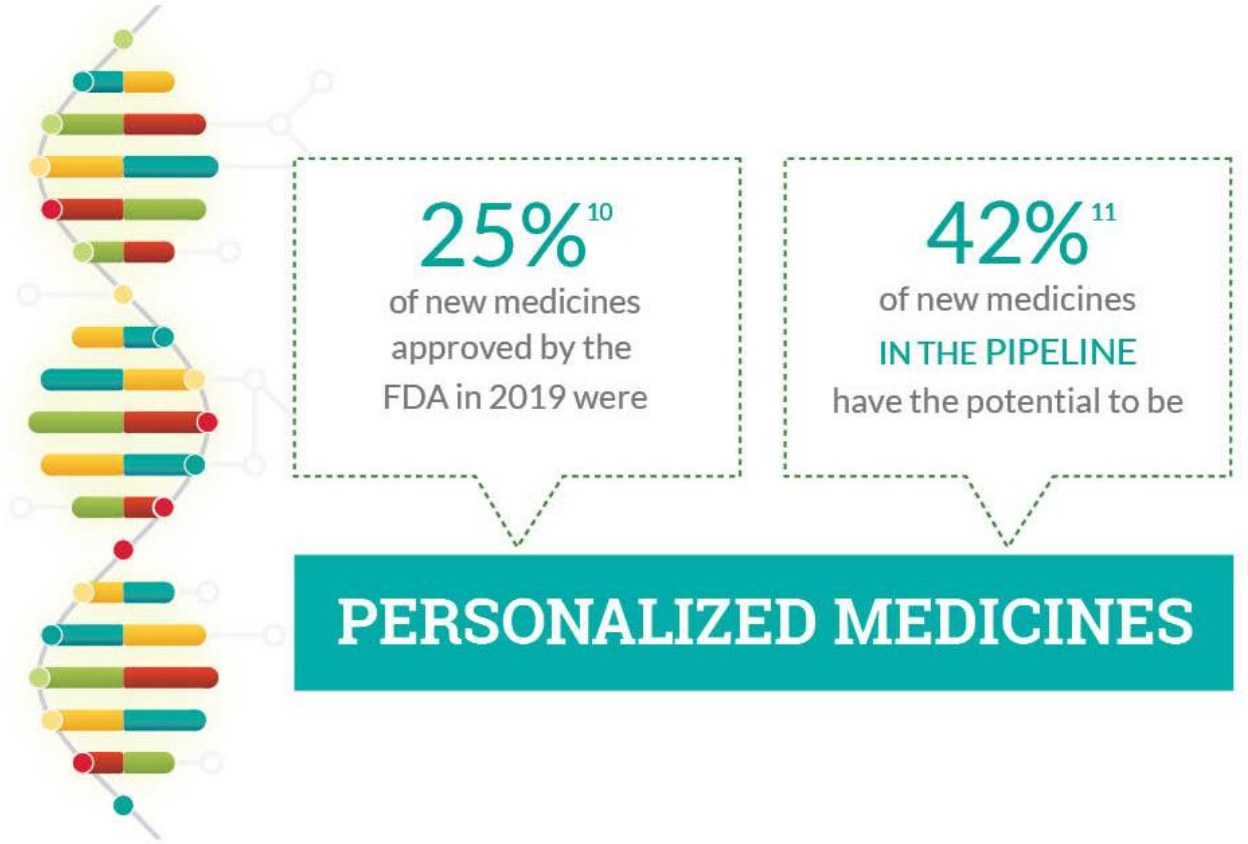
Technological advances are increasing access to clinical trials, accelerating biopharmaceutical R&D of new treatments, enabling efficient information exchange, enhancing clinical decision-making, generating information that is important to patients, and furthering patient engagement and safety.



Source: Adapted from Avalere Health[9]

Biopharmaceutical Companies Are Committed to Advancing Personalized Medicine

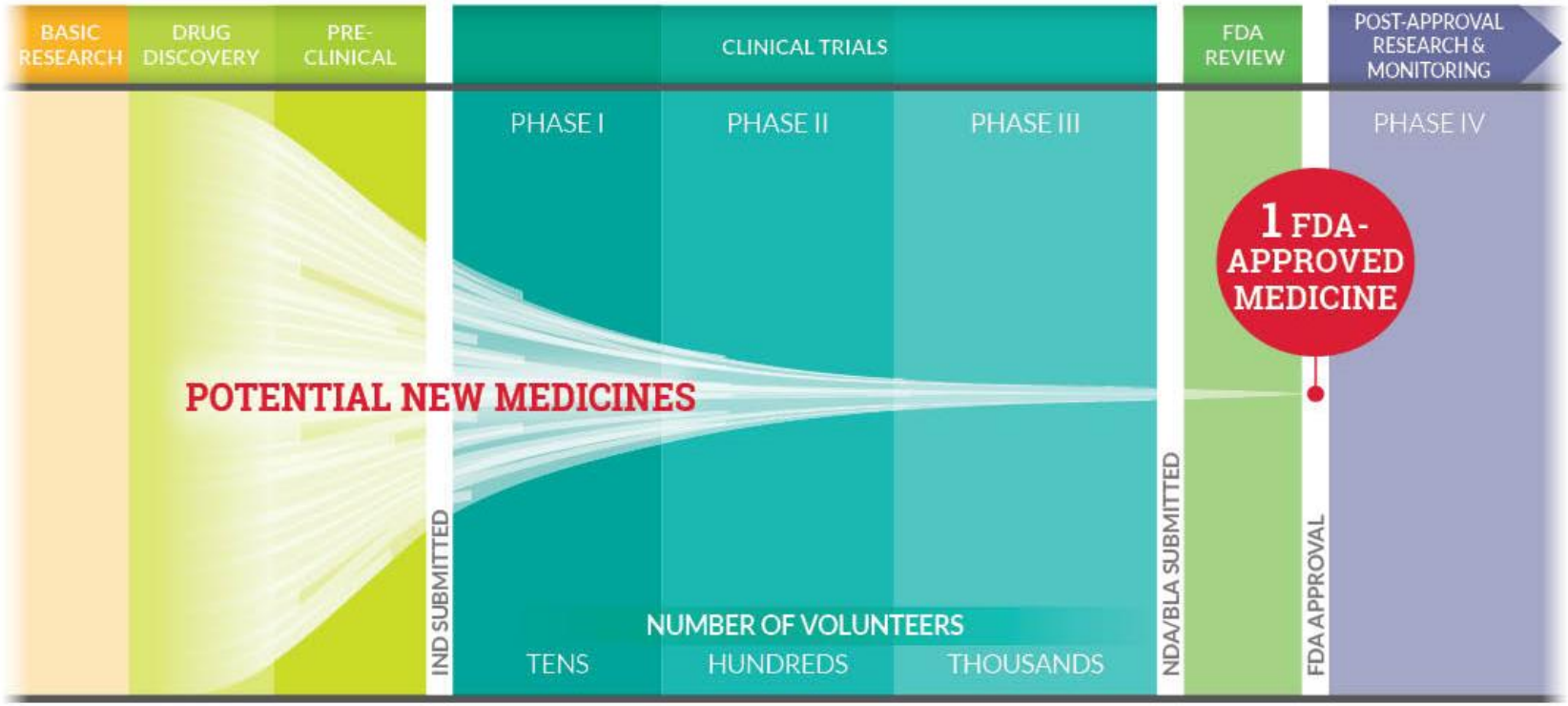
The transformation of health care from a one-size-fits-all approach to a targeted one that employs each patient's own genetic information is advancing.



Sources: Personalized Medicine Coalition[10]; Tufts CSDD[11]

The R&D Process for New Drugs Is Lengthy and Costly, With High Risk of Failure

From drug discovery through FDA approval, developing a new medicine takes, on average, 10 to 15 years and costs $2.6 billion.* Less than 12% of the candidate medicines that make it into Phase I clinical trials are approved by the FDA.

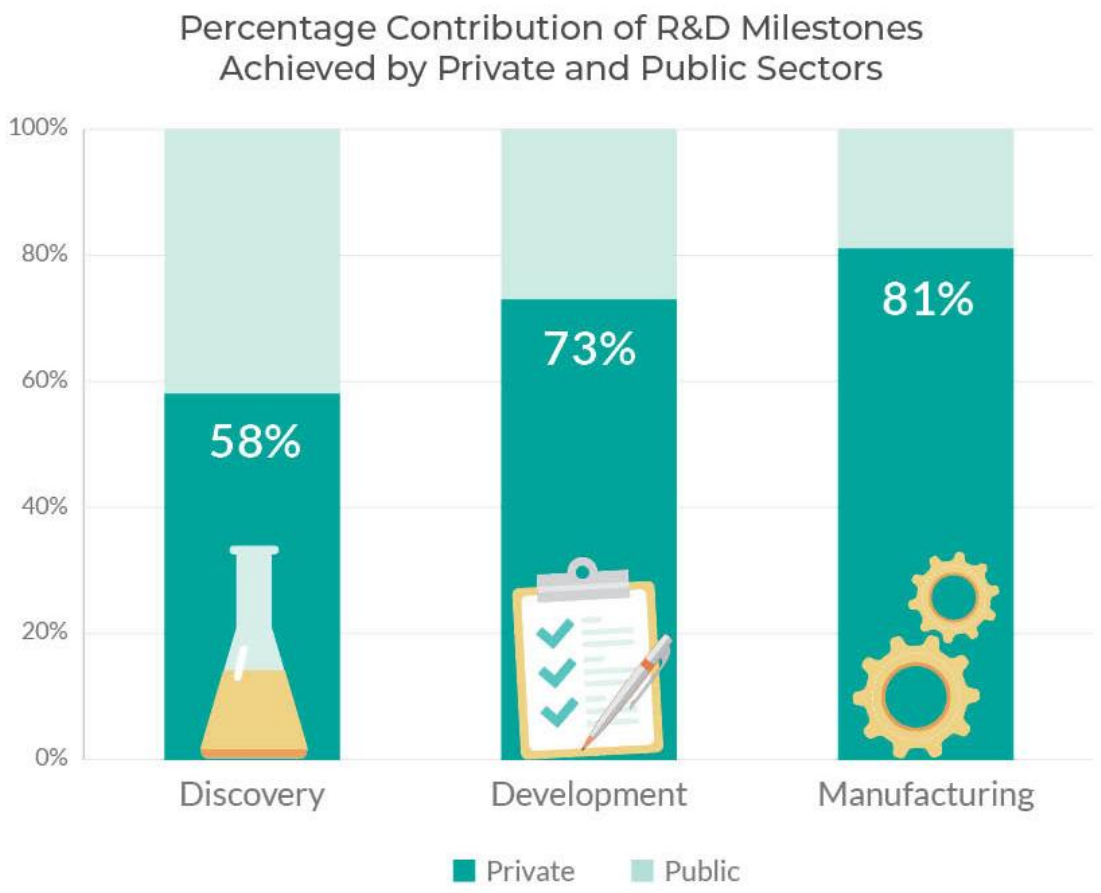


Key: IND=Investigational new drug application, NDA=New drug application, BLA=Biologics license application

*The average R&D cost required to bring a new FDA-approved medicine to patients is estimated to be $2.6 billion over the past decade (in 2013 dollars), including the cost of the many potential medicines that do not make it through to FDA approval.

Sources: PhRMA adaptation of DiMasi JA et al[12]; Tufts CSDD[13]; FDA[14]

Private Sector Scientific and Industrial Expertise Is Required to Develop and Manufacture New Medicines



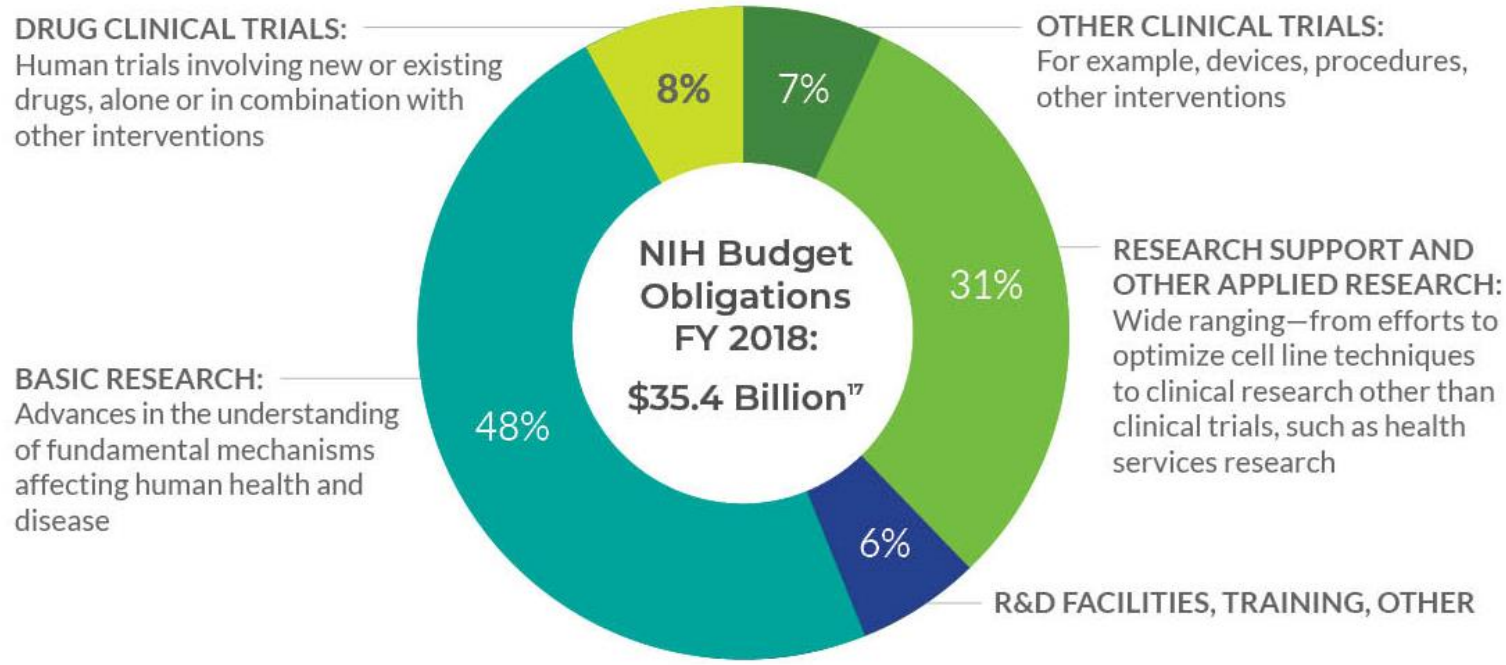
Source: Chakravarthy R et al[15]

Biopharmaceutical Industry Does the Majority of Research to Advance Basic Science Into New Medicines

The biopharma industry invested **$102 billion** in R&D in 2018, **100%** of which was focused on drug development.

The entire NIH budget in FY 2018 was **$35.4 billion,** only **8%** of which was focused directly on research related to drug development.

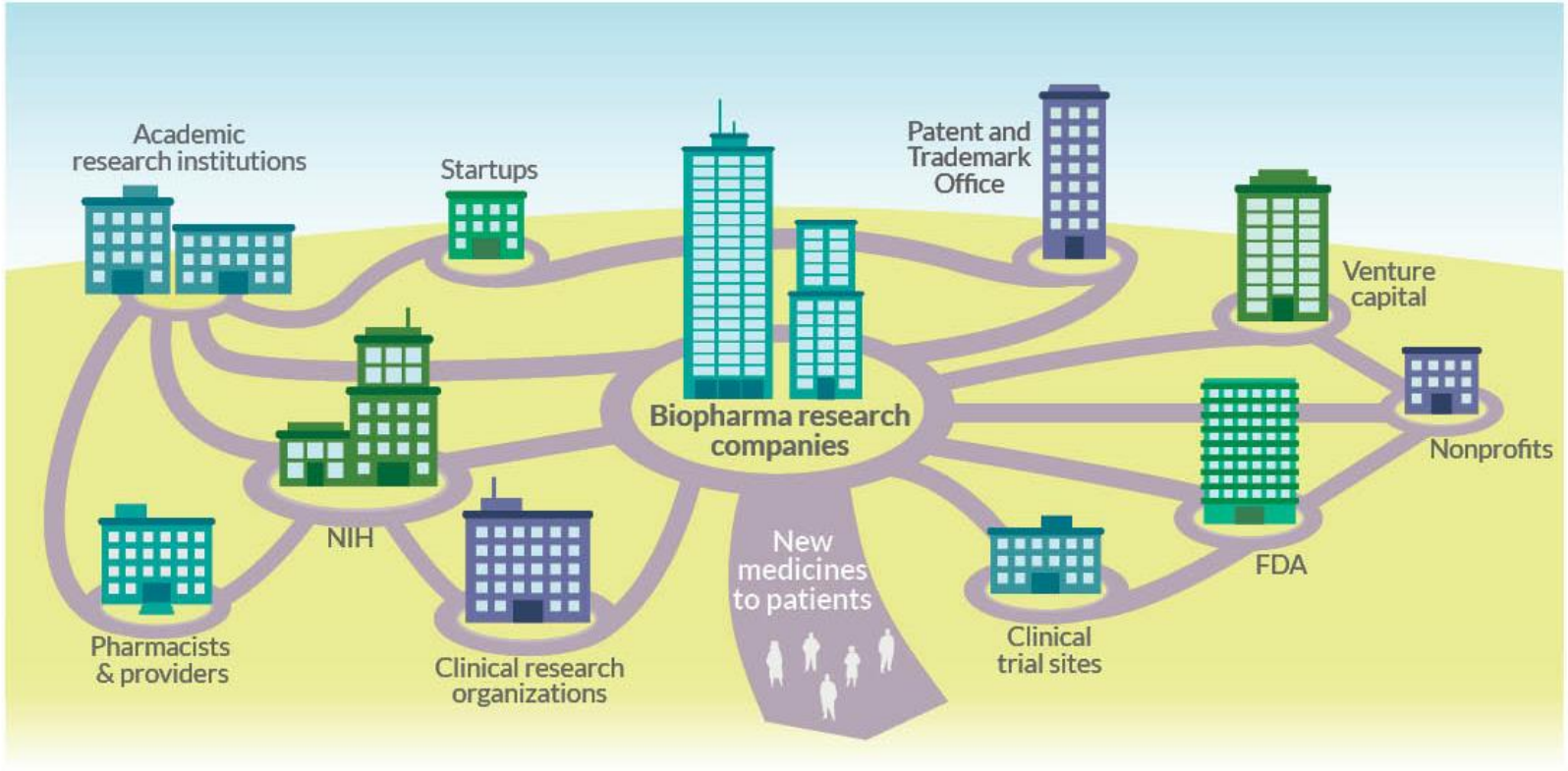
The NIH Does Research in a Variety of Important Areas Complementary to Drug Development



*NIH budget refers to fiscal year 2018. Biopharmaceutical investment refers to 2018 calender year.

Innovative Biopharmaceutical Companies Sit at the Heart of a Dynamic R&D Ecosystem in the United States

The vibrant US biomedical R&D ecosystem is critical in bringing new medicines to patients and maintaining US leadership in biopharmaceutical R&D.



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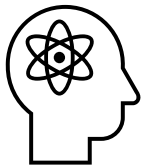
An Effort to Enhance Clinical Trial Diversity

What does it Mean for Patients?

Systemic Barriers to Clinical Trial Participation Cannot Be Solved Overnight or by One Entity Alone

Limited public awareness and understanding of clinical trials

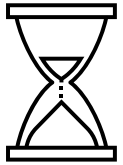


Limited access to trials sites in underrepresented communities

Lack of diverse investigators and staff

Lack of robust data on disease incidence in underserved communities

Financial and time burden that trials can place on volunteers



Biopharmaceutical Industry Is Committed to Increasing Diversity in Clinical Trials:

- Enhance **education** about the role of clinical trials
- Increase **awareness** and **lower burden** on participants
- **Broaden eligibility** criteria when scientifically and clinically appropriate
- Leverage **real-world data** to better understand effects in diverse populations
- Adopt **patient-centric approaches** / patient perspectives

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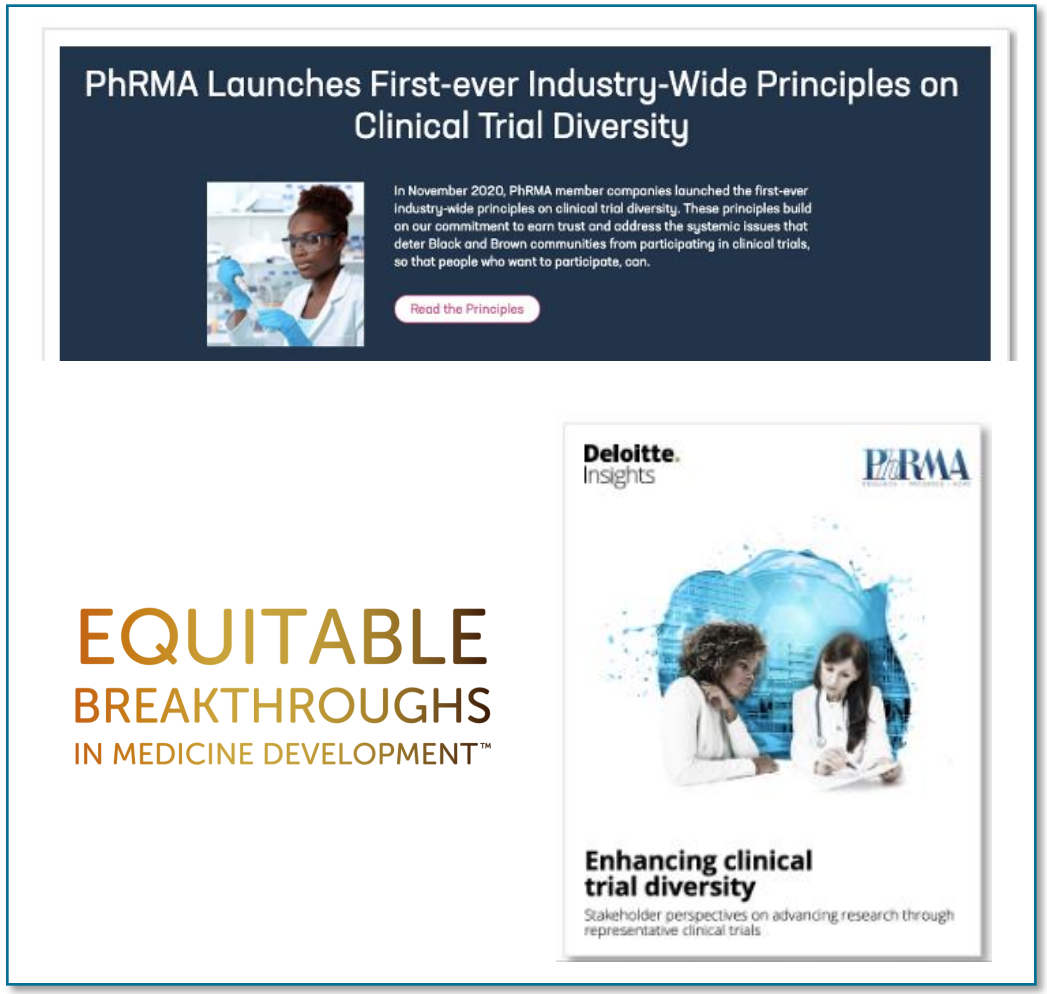
Enhancing Clinical Trial Diversity

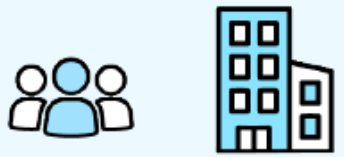
PhRMA has engaged member companies and external stakeholders on a renewed, industry-wide effort to enhance clinical trial diversity.

- **October 2020:** Voluntary industry development and adoption of Clinical Trial Diversity Principles
- **June 2021:** 500+ participants, 150+ organizations joined Clinical Trial Diversity stakeholder workshop
- **November 2021:** Release of Clinical Trial Diversity Insights Report
- **December 2021:** Proposal accepted for Learning Phase supporting community-based infrastructure
- **July 2022:** Launch of *Equitable Breakthroughs in Medicine Development*



Benefits of a community-based infrastructure for clinical trials

Changing the Paradigm

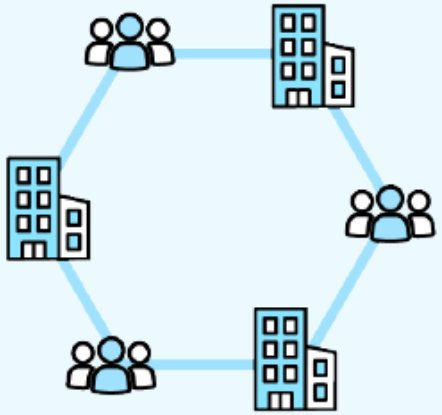


Disparate sites at varying levels of readiness / community connectiveness

Create an Infrastructure that Provides...

- A network
- Communications
- Community relationships
- Ongoing site training
- Sustainable support
- Standardized platforms / metrics

A sustainable community-based infrastructure supporting multiple touchpoints / sites dedicated to CT diversity



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Equitable Breakthroughs in Medicine Development

(EQBMED) is an industry-wide, community-based effort focused on **supporting sites and patients in underrepresented communities** to enhance clinical trial diversity in a sustainable way.

EQUITABLE BREAKTHROUGHS IN MEDICINE DEVELOPMENT™

Led by:

MOREHOUSE SCHOOL OF MEDICINE

Yale SCHOOL OF MEDICINE

RCMI COORDINATING CENTER

VANDERBILT UNIVERSITY MEDICAL CENTER

Funded by a grant from PhRMA

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Equitable Breakthroughs In Medicine Development Goals

1. Show proof of concept for a comprehensive, collaborative network of sustainable, connected, community-based sites supporting clinical trial diversity in underserved communities.

2. Partner with trusted messengers and community leaders to raise education, awareness and support for clinical trial participation.

3. Provide the resources and technical support for local sites to be successful, sustainable, and thrive.

4. Build training opportunities and mentorship for investigators and staff.

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Additional partners will support core functions of pilot infrastructure

WHO: Community Organizations, Health Systems, Technical Experts, Academia, Industry

WHERE: Partners from across the United States with an initial trial site focus in the Southeast and Southwest

Equitable Breakthroughs in Medicine Development

Next step will be:

- ✓ Establishing Advisory Committees and onboarding additional partners
- ✓ Selecting initial clinical trial sites for the 18-month Learning Phase
- ✓ Soliciting clinical trial protocols and proposals to the infrastructure for consideration

PhRMA.org/Equity

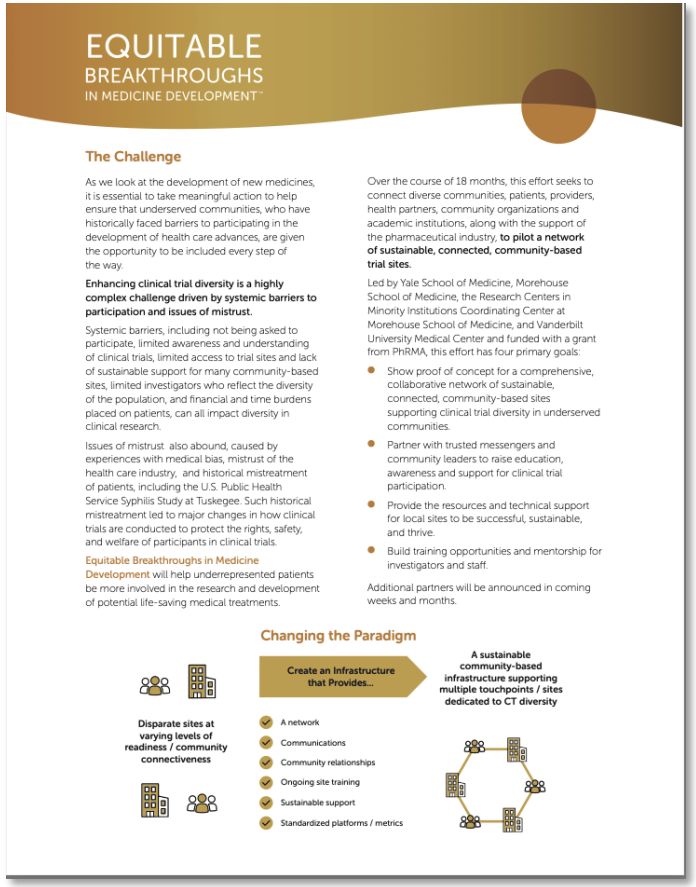
Improving Clinical Trial Diversity is Critical to Health Equity

Enhancing clinical trial diversity is a highly complex challenge that requires a community-based, multi-stakeholder approach.

Learn more about PhRMA's efforts to address the systemic barriers that can deter underserved communities from participating in clinical trials, so that people who want to participate, can.

Equitable Breakthroughs in Medicine Development

About This Effort



EQUITABLE BREAKTHROUGHS IN MEDICINE DEVELOPMENT™

The Challenge

As we look at the development of new medicines, it is essential to take meaningful action to help ensure that underserved communities, who have historically faced barriers to participating in the development of health care advances, are given the opportunity to be included every step of the way.

Enhancing clinical trial diversity is a highly complex challenge driven by systemic barriers to participation and issues of mistrust.

Systemic barriers, including not being asked to participate, limited awareness and understanding of clinical trials, limited access to trial sites and lack of sustainable support for many community-based sites, limited investigators who reflect the diversity of the population, and financial and time burdens placed on patients, can all impact diversity in clinical research.

Issues of mistrust also abound, caused by experiences with medical bias, mistrust of the health care industry, and historical mistreatment of patients, including the U.S. Public Health Service Syphilis Study at Tuskegee. Such historical mistreatment led to major changes in how clinical trials are conducted to protect the rights, safety, and welfare of participants in clinical trials.

Equitable Breakthroughs in Medicine Development will help underrepresented patients be more involved in the research and development of potential life-saving medical treatments.

Over the course of 18 months, this effort seeks to connect diverse communities, patients, providers, health partners, community organizations and academic institutions, along with the support of the pharmaceutical industry, **to pilot a network of sustainable, connected, community-based trial sites.**

Led by Yale School of Medicine, Morehouse School of Medicine, the Research Centers in Minority Institutions Coordinating Center at Morehouse School of Medicine, and Vanderbilt University Medical Center and funded with a grant from PhRMA, this effort has four primary goals:

- Show proof of concept for a comprehensive, collaborative network of sustainable, connected, community-based sites supporting clinical trial diversity in underserved communities.
- Partner with trusted messengers and community leaders to raise education, awareness and support for clinical trial participation.
- Provide the resources and technical support for local sites to be successful, sustainable, and thrive.
- Build training opportunities and mentorship for investigators and staff.

Additional partners will be announced in coming weeks and months.

Changing the Paradigm

Disparate sites at varying levels of readiness / community connectivity

Create an Infrastructure that Provides...

- A network
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A sustainable community-based infrastructure supporting multiple touchpoints / sites dedicated to CT diversity

Industry Efforts to Enhance Clinical Trial Diversity

JULY 2022

PhRMA
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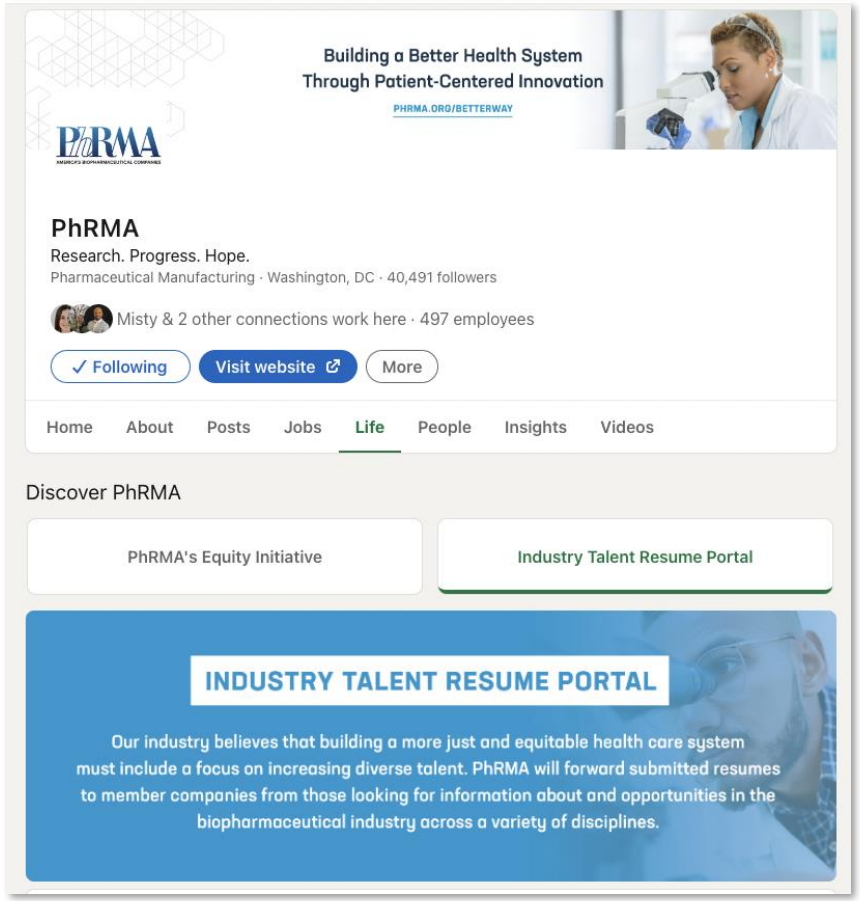
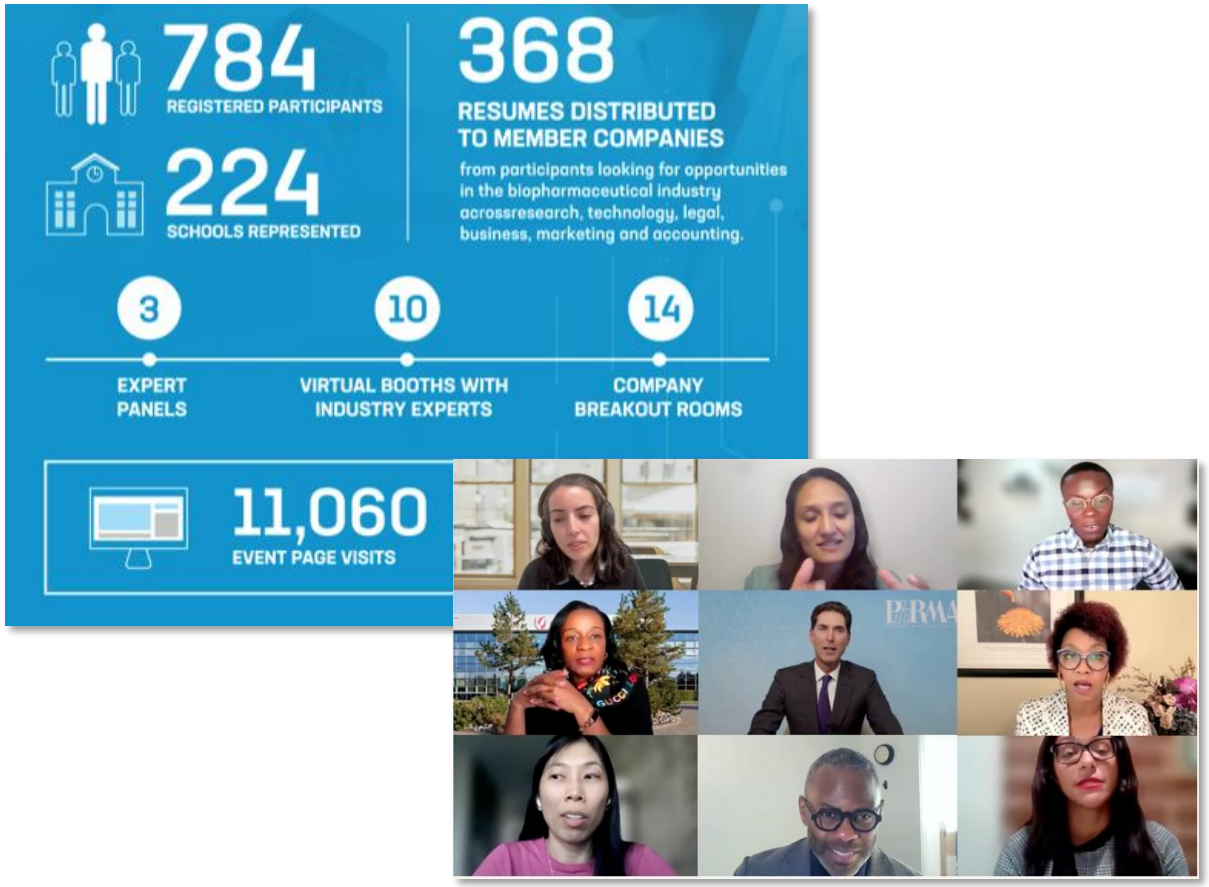
Handouts, video clips, reports and more

Creating Additional Pathways to Our Industry



LinkedIn Community



Annual Grad Summit / Career Expo

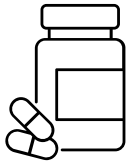
PhRMA Created the Medicine Assistance Tool (MAT) To Help Patients Navigate Medicine Affordability

A search engine to connect patients with

900+

assistance programs offered by biopharmaceutical companies, including some free or nearly free options

Resources to help patients navigate their insurance coverage



Links to biopharmaceutical company websites where information about the cost of a prescription medicine is available

Learn more at www.MAT.org