Innovation in R&D for Prevalent Chronic Disease

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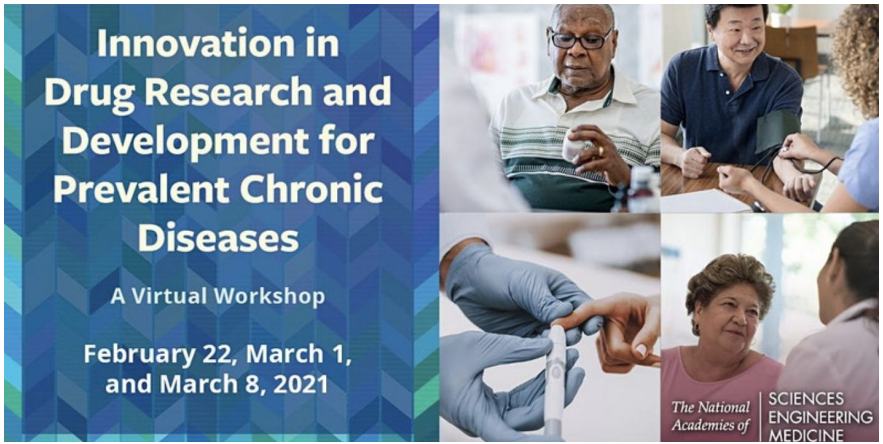
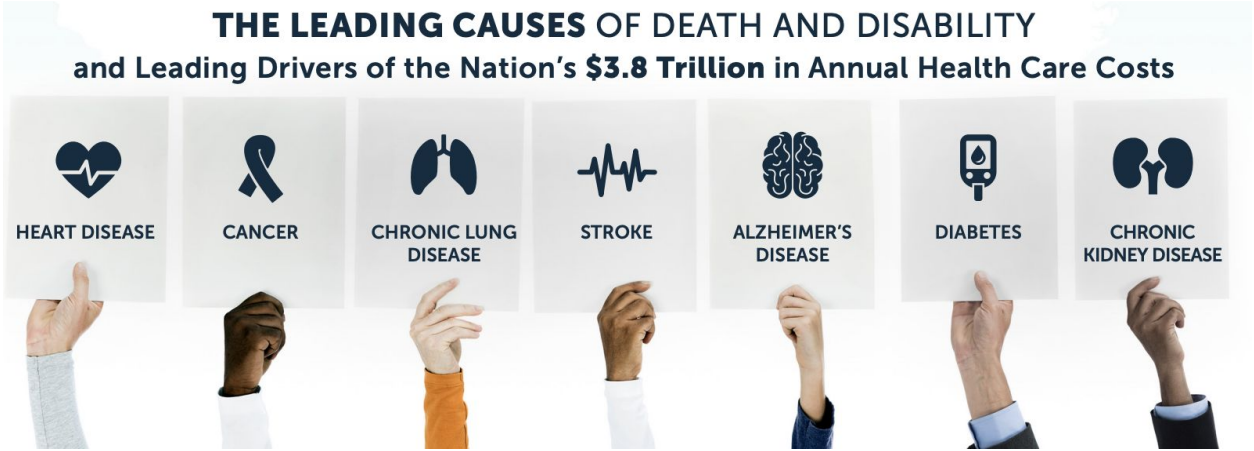


6 IN 10
Adults in the US
have a **chronic disease**

4 IN 10
Adults in the US
have **two or more**

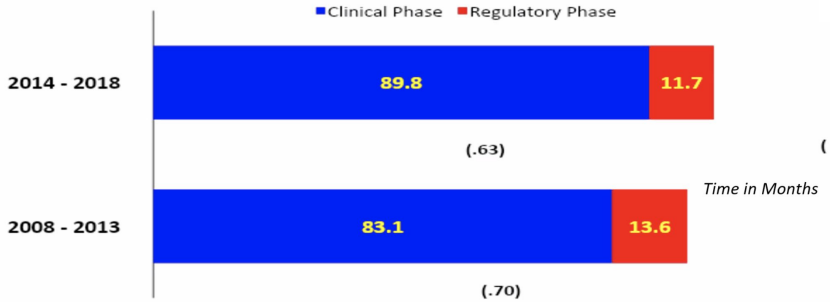
Source: CDC website



Source: CDC website

Challenges: Duration and Costs are Increasing

Historical Clinical & Regulatory Durations



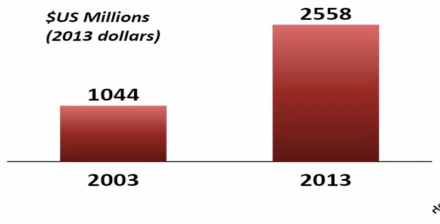
Source: Tufts CSDD, 2020. N = 377 new drugs and biologics approved by CDER (FDA) between 2008 and 2018

Tufts Center for the Study of Drug Development

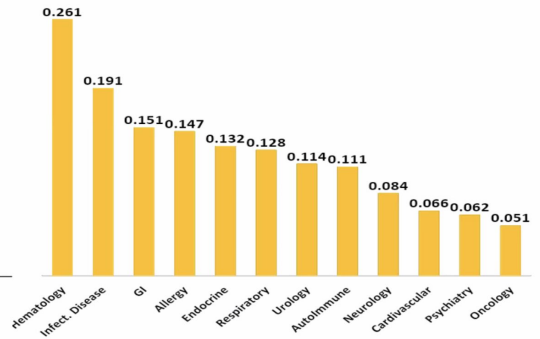
Extremely High Risk and High Cost

Capitalized Cost to Develop a Successful Drug

- 26% Direct Costs
- 18% Time-Based
- 56% Cost of Failure



Probability of Achieving Regulatory Approval by Disease



Source: Tufts CSDD

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Challenges: Increased Complexity of Trials and Investment Areas

Complexity and Customization

Phase III Pivotal Trials *(means per protocol)*	2005	2011	2020	15-Year CAGR
Total Endpoints	7	13	19	6.9%
Total Eligibility Criteria	31	34	30	-0.2%
Total Procedures	110	187	263	5.9%
Number of Substantial Amendments	2.0	2.3	3.4	3.6%
Total Countries	6	9	15	6.3%
Total Investigative Sites	40	65	87	5.3%
Number of data points *(in 000s)*	494	924	3,560	14.1%

Source: Tufts CSDD

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IPO's by Therapeutic Area

THERAPEUTIC FOCUS	2021	2020	2019	2018
Cancer	9	34	15	17
Rare	1	12	3	8
Immune	1	6	4	5
CNS	0	4	6	1
Infectious	0	6	0	5
Other	3	8	10	8

"Other" includes liver, eye, heart, gastrointestinal, metabolic, ear, skin and kidney.

Source: Biopharm Dive

Challenges: Lack of Diversity in Clinical Trials and Growing Disparity in Health Equity

FDA STATEMENT

FDA Offers Guidance to Enhance Diversity in Clinical Trials, Encourage Inclusivity in Medical Product Development

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For Immediate Release: November 09, 2020

Statement From: Commissioner of Food and Drugs - Food and Drug Administration (December 2019 - January 2021)
Stephen M. Hahn M.D.

Opportunities are Many: $, Tech, Regulatory Flexibility and COVID learnings

BIOBUSINESS BRIEFS · 20 JANUARY 2021

2020 biotech IPOs shatter all the records

ICH
harmonisation for better health

COVID-19: A Catalyst to Accelerate Global Regulatory Transformation

Jerry Stewart[1,*], Peter Honig[1], Lina AlJuburi[2], Deborah Autor[3], Susan Berger[4], Patrick Brady[5], Helen Fitton[6], Carlos Garner[7], Michael Garvin[3], Mathias Hukkelhoven[1], Robert Kowalski[8], Sandra Milligan[2], Liza O'Dowd[9], Edward Reilly[10], Khyati Roberts[11], Andrew S. Robertson[10], Mark Taisey[12], Roopal Thakkar[9], Karin Van Baelen[9] and Max Wegner[5]

A global crisis the magnitude of coronavirus disease 2019 (COVID-19) can transform drug development and review. It has exposed vulnerabilities and inadequacies in the global healthcare ecosystem as well as spurred innovation, rapid adaption of novel solutions, and unprecedented collaboration among global regulatory agencies, sponsors, and researchers. For the continued and future benefit of patients, it is imperative that all stakeholders leverage the solutions, learnings, and momentum catalyzed by this crisis to advance regulatory science in the drug development process.

Focus Areas for Progress in Chronic Disease

1. **Collaborative Investments:** Technology and Biopharma improving lives together, partnering with regulators on frameworks

2. **Early Disease:** Clinical Trials and Regulatory Frameworks targeting early disease treatment - novel trials and endpoints leveraging strong regulatory framework for decision making

3. **Diversity:** As sponsors and regulators - we must take action now

4. **Innovative Trial Approaches:** Industry and regulators are partnering to enable novel, valid approaches for regulatory decision-making

5. **Incorporate Patient Voice:** With a patient focused approach, we can run more patient-centered trials, measuring outcomes that matter for their health

Doing now what patients need next