Catenion

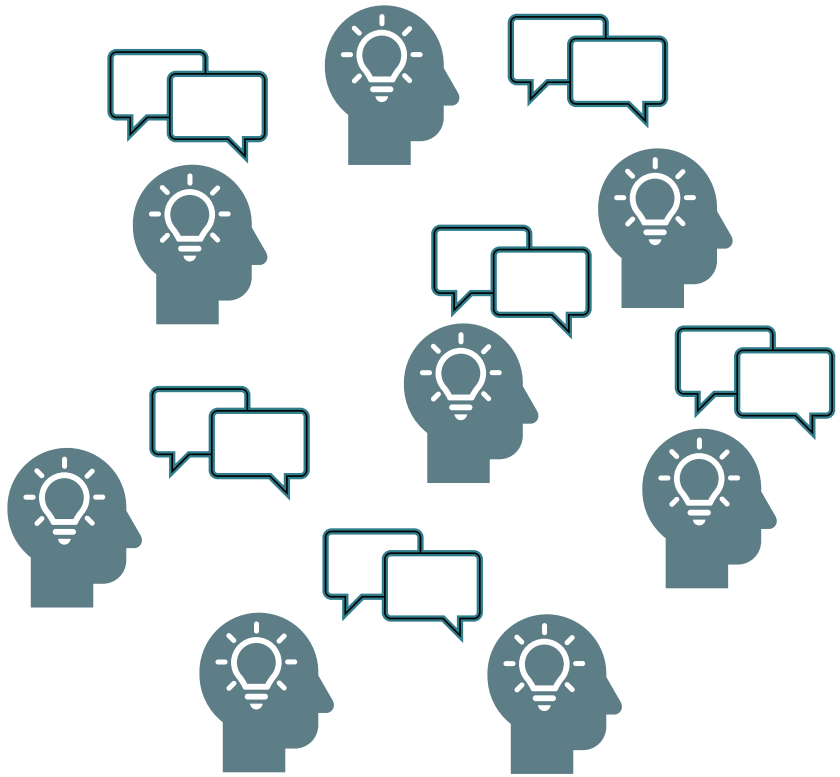
Workshop: Financing Innovation in Women's Health

Moderated by Catenion

7 June 2023

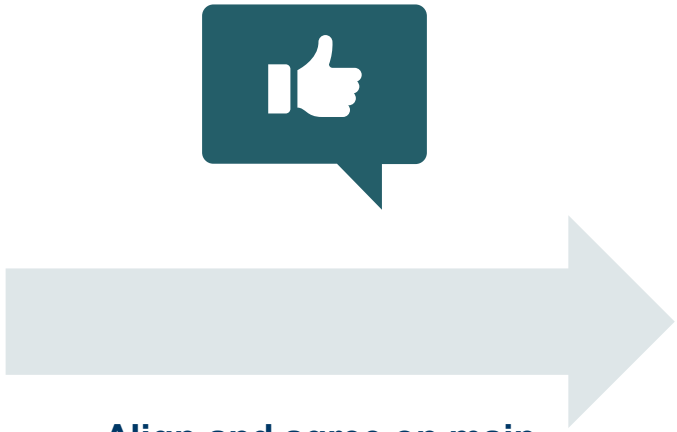
This workshop does not aim at listing the challenges we all know well, but rather on exchange and document insights and actionable solutions

Workshop Goals



Sharing thoughts and ideas

Enter the “What if” domain



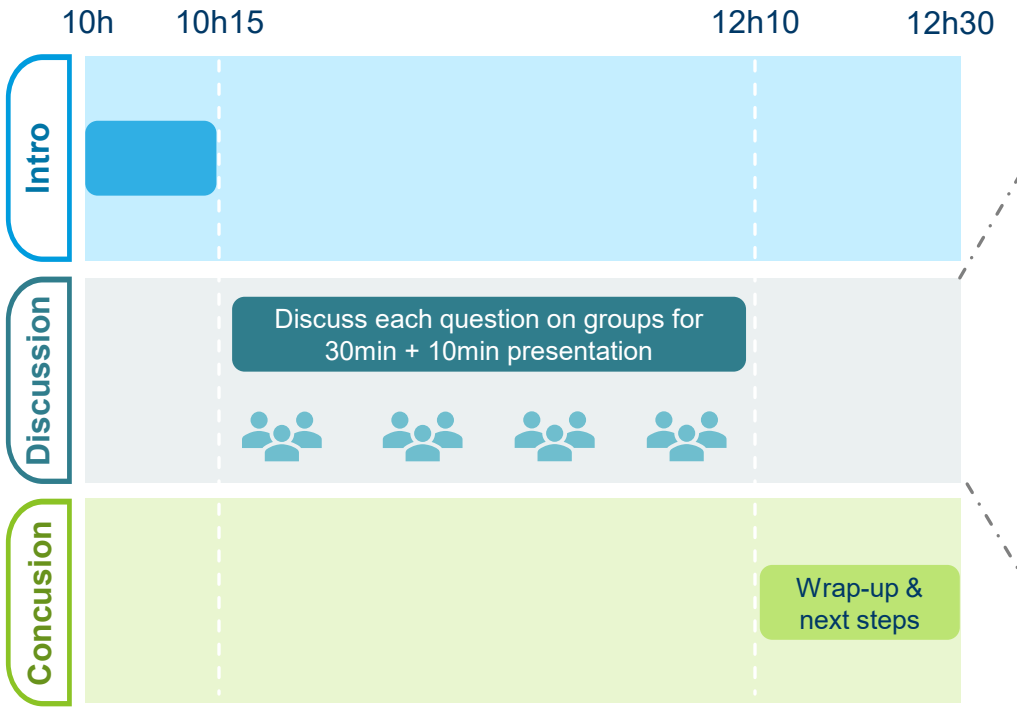
Align and agree on main insights and suggestion to move forward

“How to finance innovation in Women’s Health?”

Prepare a white paper (post-workshop) with insights actionable solutions

This workshop will mainly focus on the discussion of the 3 key questions that can shape how we understand innovation in Women's' health

Workshop Modus Operandi



Main Questions for Discussion

1. How to define innovation in Women's Health?
   - What is the rationale for investing in Women's Health?
   - How to create a positive business case?
2. What strategies can be put in place to share the burden of development for novel innovative medicines in women's health?
   - What initiatives are already in place for WH?
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   - For the push-and-pull between Basic & Applied Research: What needs to happen for academia to play a larger role in biopharma innovation?

***Disclaimer**: focus on financing of disease-modifying treatments*

Agenda

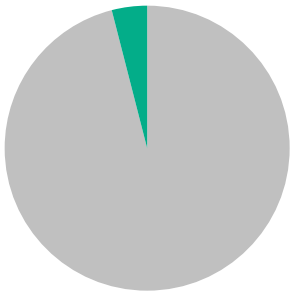
- **Introduction**
- Case Studies
- Discussion

In Women's Health, the roots of disparity are deep both in Funding and R&D

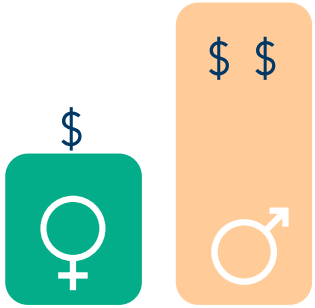
Introduction

Funding

	% Females affected	% Funding on female-focused projects
Alzheimer's disease	~66% of Alzheimer's patients are females	12% of the $2.4 bn NIH funding on Alzheimer's disease
Coronary artery disease	55% higher chance of death following heart attack for females	4.5% of the $444 mn NIH funding on coronary artery disease
Autoimmune Diseases	78% of US citizens with autoimmune diseases are females	7% of the $85 mn NIH Rheumatoid Arthritis budget

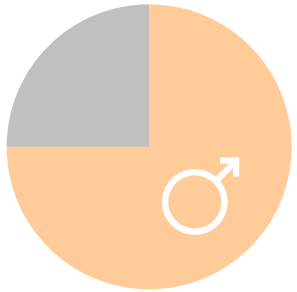


***4%** of **healthcare R&D spending** in the US goes directly to Women's Health (1% outside of oncology)*



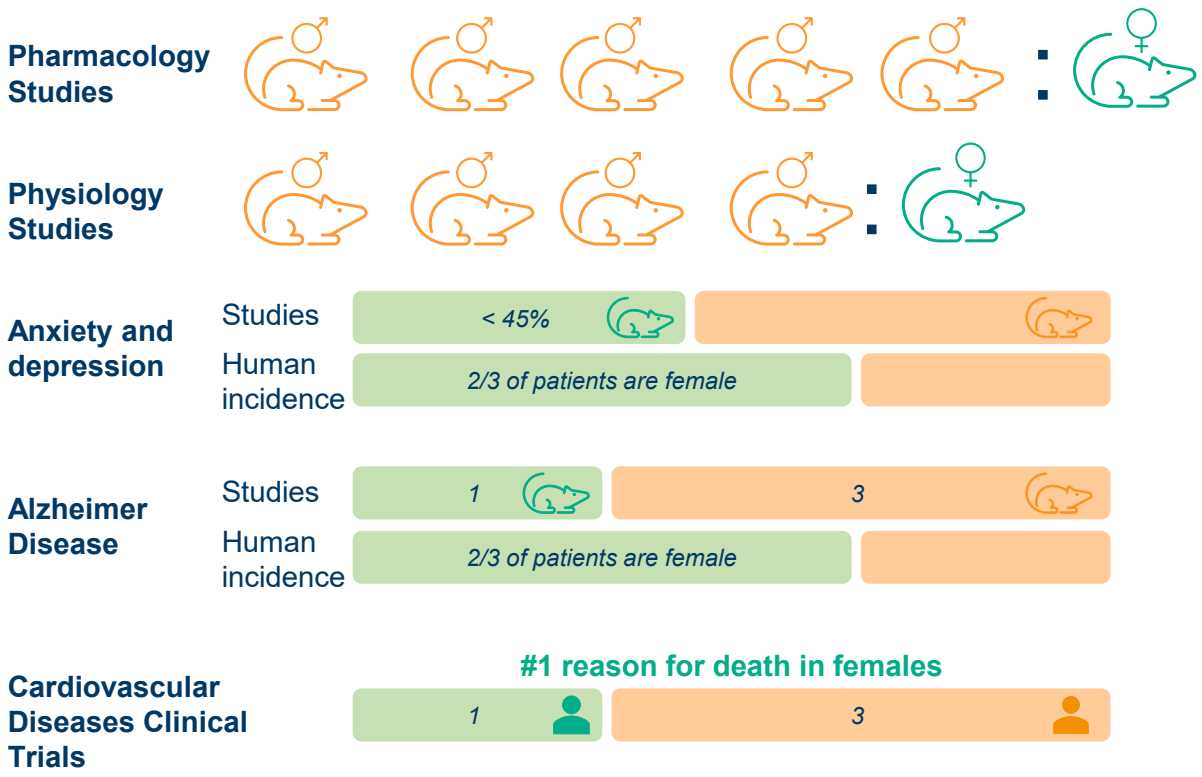
2x More funding

Male prevalent diseases vs female prevalent diseases



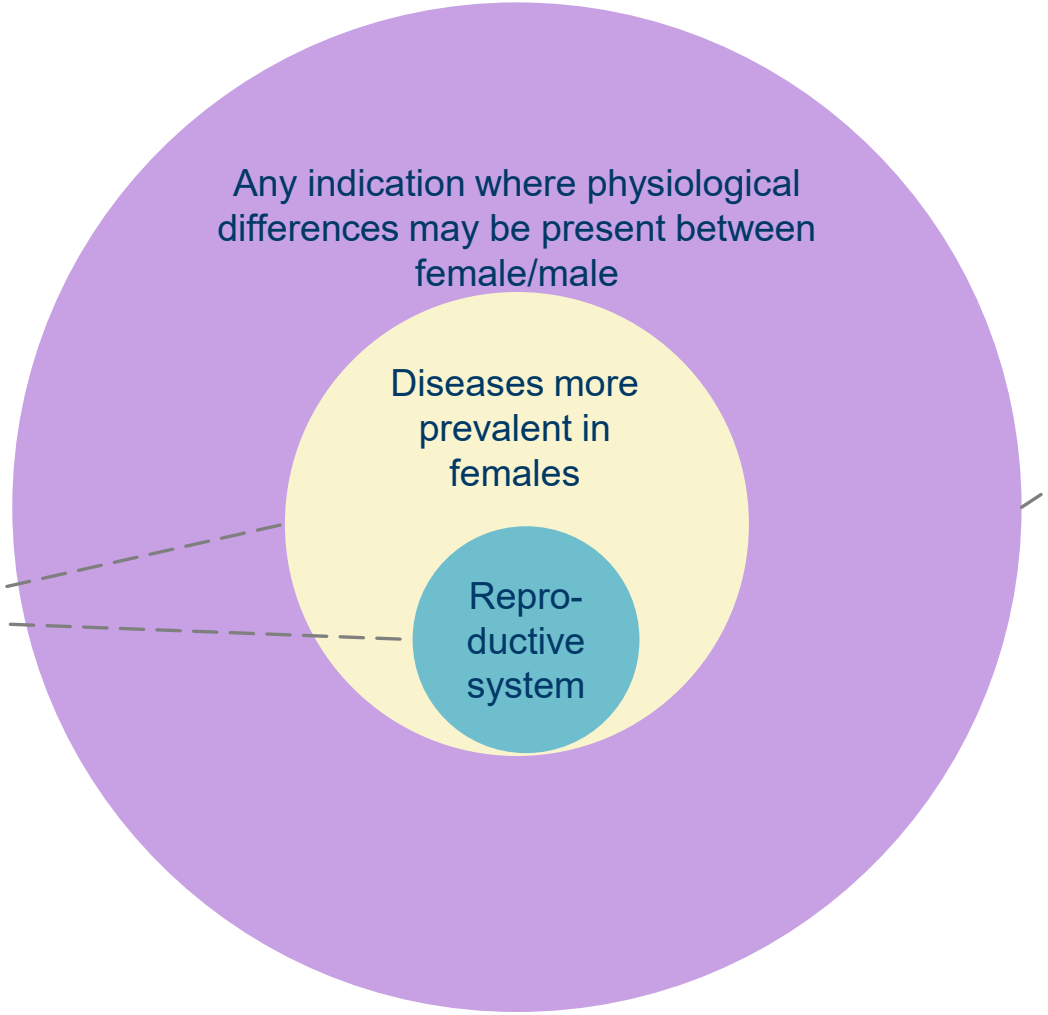
~75% of the cases where a disease afflicts primarily one gender, the funding pattern favors males

Research & Development



How can Women's Health be better defined? How to leverage the most logical Biomarker to be used in drug development?

Introduction



These are the most obvious choice when defining Women's Health

But what about the ~50% of patients in all other indications that are females?

"Everyone is speaking about personalized medicine, having personalized approaches for every individual (…) I think having a step before there, maybe **stratifying the population into male and female, and optimizing treatment strategies** there, is an important step."

Dr. Marcus Altfeld, an immunologist at the Heinrich Pette Institute in Hamburg

If we trace back the steps from the goal of financing innovation in, the two founding steps are Policy Making and Public Investment

Perspective: How to get there?

Catenion's perspective - TBD

Public Investment
Public investment into Women's Health space through research grants, formation of sex-specific databases

Clinical Validation
Research partnerships & clinical PoC data creation by biopharma

Financing
Financing innovation in Women's Health mainly through private investment

1 2 3 4 5 6

Policy Making
Effective policy making that increases awareness of Women's Health indications with high unmet needs

Basic Research
Understanding of the pathophysiology in depth through thorough preclinical research

Value Creation
Creation of intriguing value propositions that attract private investment

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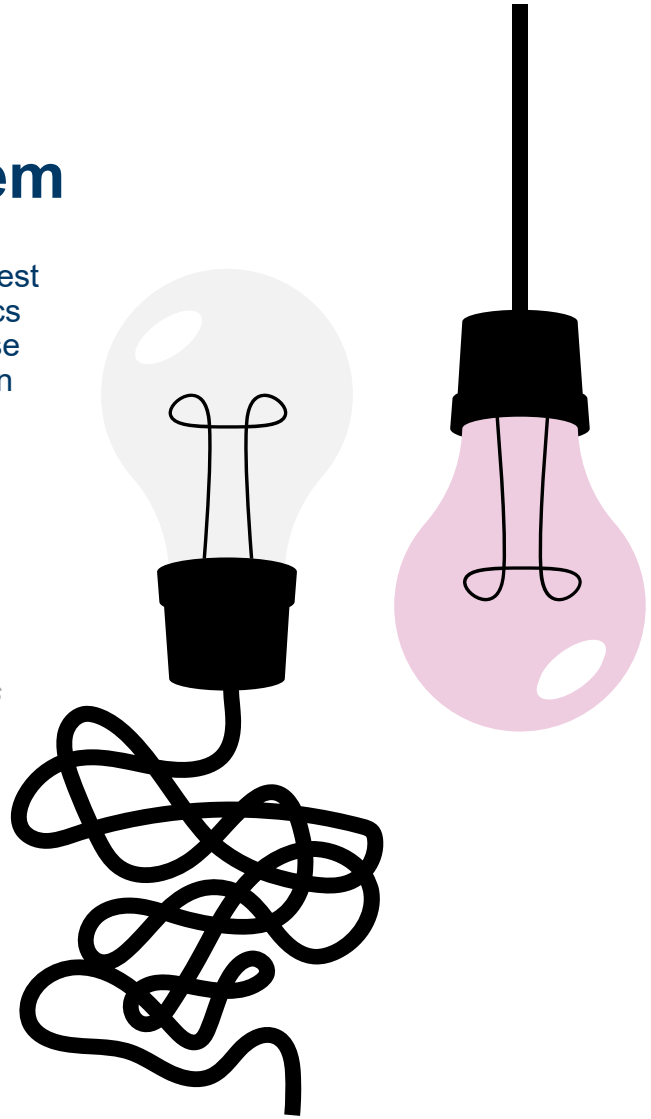
Public Investment and Policy Makers were the drivers of the change in the investment paradigm in Orphan Diseases

Case Study: Orpah Drugs

Problem

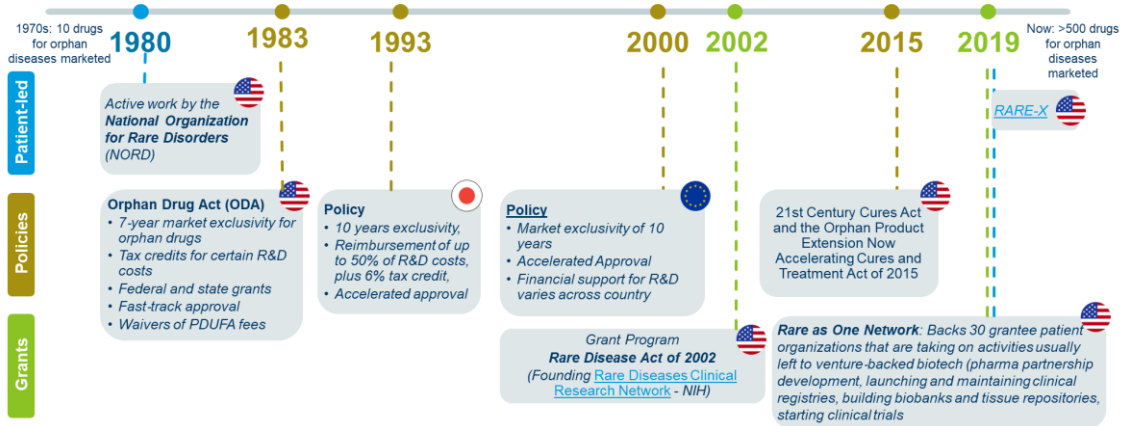
- For many years, biotechnology and pharmaceutical companies did not invest much in developing drugs and biologics for rare diseases or conditions because "there [was] no reasonable expectation [that] the sales of the drug[s would] recover the costs".
- But that changed…

Parallelism in WH → positive *business case is challenged due to the high generics use and consequent pricing issues*



Solution

- On top of the rare disease patient advocacy groups which formed a coalition that became the National Organization for Rare Disorders (NORD) and in the 1980s; Senator Orrin Hatch and with Representative Henry A. Waxman, were instrumental in passing the Orphan Drug Act in 1983.
- Main drivers: policy makers & regulators (US, EU, JP), patient organizations, public finding agencies (e.g., NIH)
- Overall, 1983 ODA resulted in the approval of over 650 orphan drugs



Could such framework work to incentivize repurposing of existing and funding of new drugs for women specifically?

Source:

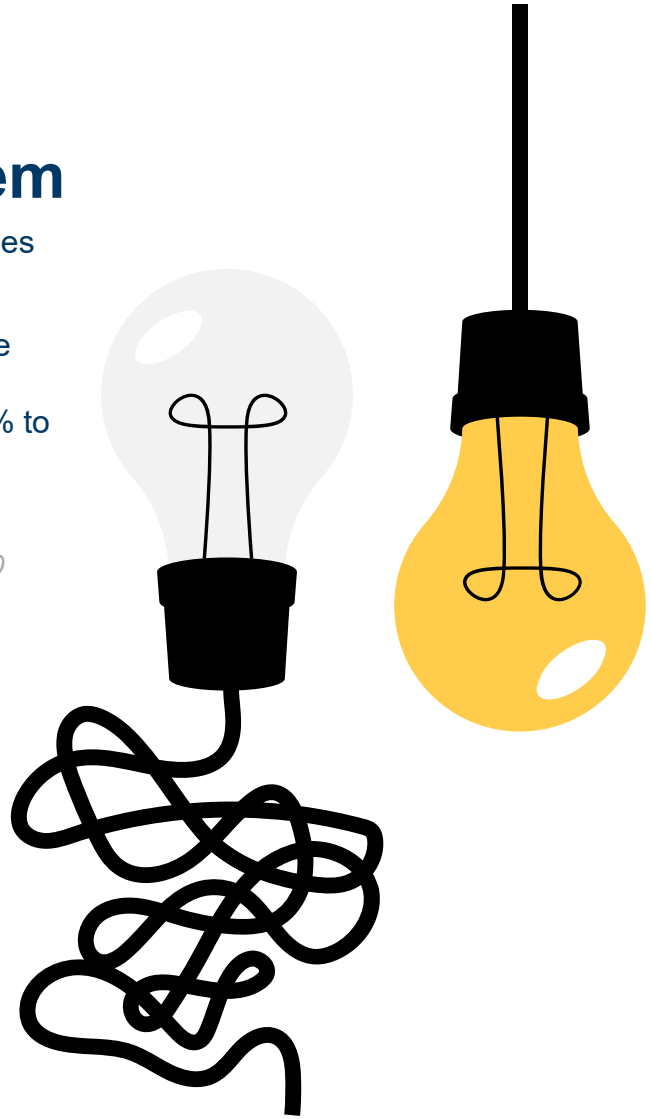
BPCA and PREA were established for similar reasons that we observe on women today: safety and effectiveness differences

Case Study: Pediatrics

Problem

- There are clear differences in responses to drugs between children and adults, including different dosages, unique pediatric adverse events, and even the inability to demonstrate effectiveness
- As a consequence, approximately 65% to 80% of drugs have not been tested in children.

Parallelism in WH → *86 of 668 drugs of the 20 most frequent treatment regimens in the US have significant sex differences in pharmacokinetics in females*



Solution

- The Best Pharmaceuticals for Children Act (BPCA) in 2002
- The Pediatric Research Equity Act (PREA) in 2003

Driver: United States Congress

Incentive	BPCA	PREA
Exclusivity Extensions	Provides a six-month extension of market exclusivity for a new drug application (NDA) if pediatric studies are conducted	
Financial Support	Offers financial support to conduct pediatric research	N/A
Regulatory Streamlining	Establishes clear guidelines and procedures for conducting pediatric studies, streamlining the regulatory process	Streamlines the review and approval process for pediatric labeling updates
Market Expansion	Allows drug developers to target pediatric populations more effectively, potentially expanding the market	
Labeling Updates	Requires submission of pediatric study data to update drug labels with pediatric-specific information	

Could such framework work to incentivize repurposing of existing and funding of new drugs for women specifically?

Source: Kwok et al., 2015; FDA's Authority to Ensure That Drugs Prescribed to Children Are Safe and Effective , 2012

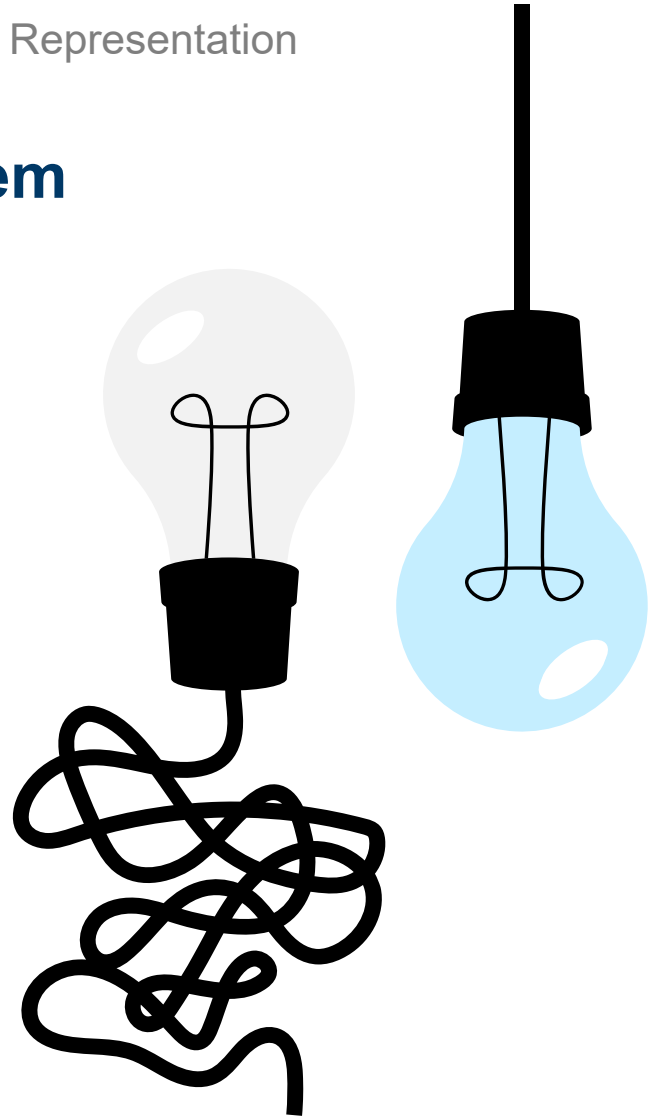
Policy and legislative measures have also been taken for for ethnic and racial under-representation in clinical trials

Case Study: Racial and Ethical Representation

Problem

Underrepresentation of certain racial and ethnic groups in clinical trials

Parallelism in WH → *females are underrepresented in preclinical & clinical trails. E.g., Cardiovascular Diseases is #1 reason for death in females but in clinical trails women are underrepresented (1Female: 3 Males)*



Source, Schick and Axelsen, 2021

Solution

Year	Legislative Act	Main Measures
2007	**FDA Amendments Act of 2007 (FDAAA)**	- Drug developers to report demographic data for subgroups of clinical trial participants - Strengthens FDA's authority to enforce requirements for diversity in clinical trials - Encourages development of drugs that address health disparities
2012	**Prescription Drug User Fee Act (PDUFA)**	- Standardized definition of underrepresented populations - Drug developers to submit diversity plans for clinical trials along with their drug applications - Financial incentives for drug developers who meet diversity targets in clinical trials - Establishes a public database of clinical trial diversity data - Requires FDA reviewers to receive training on the importance of diversity in clinical trials
2016	**21st Century Cures Act**	- Increases funding for clinical trial infrastructure in underrepresented communities - Encourages the use of patient-focused drug development methods - Requires the development of clinical trial recruitment strategies tailored to underrepresented populations - Allows for the use of real-world data to supplement clinical trials
2021	**FDA's guidance on Enhancing the Diversity of Clinical Trial Populations**	- Guidance on how to implement diversity plans in clinical trials - Establishes a diversity and inclusion advisory committee to provide guidance to the FDA - Increases transparency around the FDA's evaluation of diversity in drug approvals

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What can be done outside of the current paradigm for funding innovation in Women's Health?

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What if…

Goal: Draft a collection of actionable solutions and thoughts to increase investment in Women's Health

Question 2 AID: To be printed as a Poster (A0) for participants to add post-its

Current Initiatives to support innovation in Women's Health

Unclear pathophysiology & MoAs in disease / TA

Basic Research

Basic research on pathophysiology, targets, outcomes, etc.

Applied Research

Testing preclinical hypothesis, developing early theories for pathophysiology

Development

Preclinical PoC achieved and clinical pipeline starting to be populated by Biotech and Pharma

Commercialization / Diffusion

Clinical PoC achieved and Marketed Drugs

Understanding and Treating the disease

Regulatory agencies

Public Investors & Policy Makers & Patient Orgs

Venture Capital

Stock Market

Academic Research

Biotech & Pharma

Question 3 AID: To be printed as a Poster (A0) for participants to add post-its

What can be done to support innovation in Women's Health

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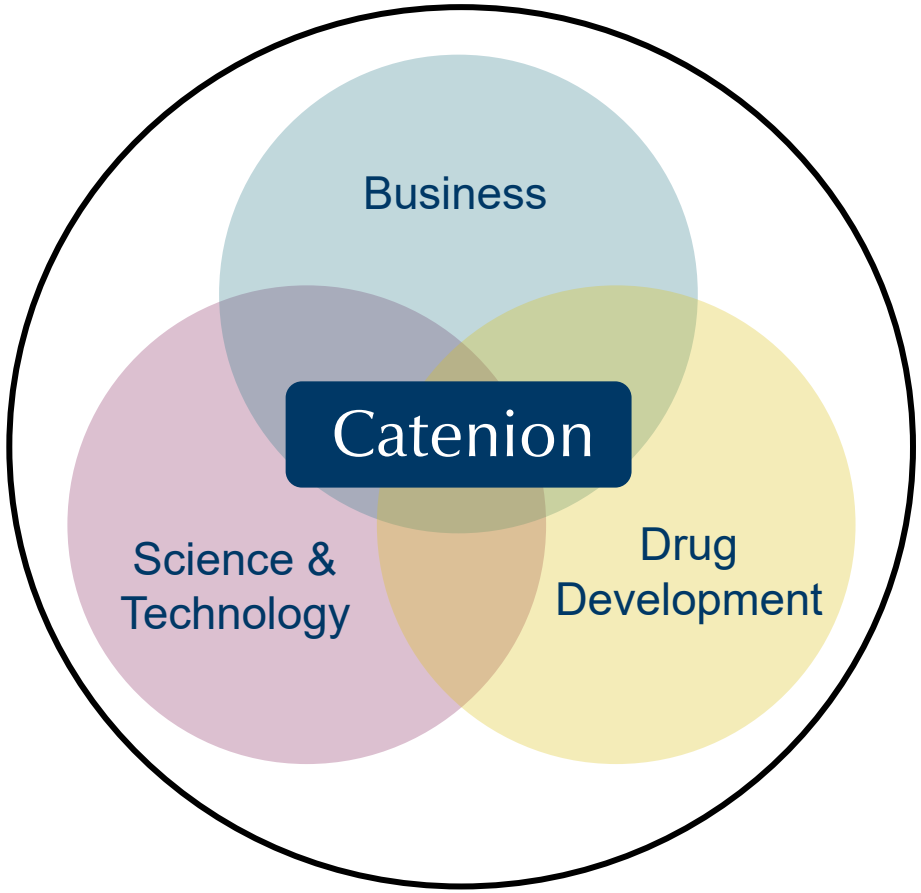
Catenion is a science-driven strategy consultancy with a proven track record of creating value for biomedical clients and patients

- Largest team exclusively focused on Biopharma R&D
- Objectivity and independent thought
- Assessed >1,500 projects often in collaboration with project teams



- Helped develop numerous marketed drugs and medical devices
- Value creation through portfolio strategy and optimization
- Trusted partners of top executives in Europe, the US and Japan

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