Embedding Diverse Patient Insights in the End-to-End Development of Medicines

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Equitable Healthcare is a Core Commitment & is Embedded in a Fit-for-Purpose Manner

As a science-driven Pharmaceutical & Diagnostics company, our work on inclusive health is focused on gaps and solutions that are related to healthcare

Our inclusive health work focuses on, but is not limited to:

- **Improving** inclusive patient representation in clinical research.
- **Generating** data to assess innovation safety and efficacy across patient sub-populations.
- **Enhancing** health literacy in key areas for scientifically representative research.
- **Driving** early screening and diagnosis.
- **Developing** integrated health solutions (e.g., subcutaneous formulations, digital monitoring) to increase healthcare accessibility for all patients in our disease areas.

Inclusive Health is about taking a holistic look at the gaps that exist within healthcare systems so that we can partner with a broad group of external partners to **co-create localised solutions focused on the unique needs of sub-populations representative of disease.**

Why Patient Inclusivity is Critical in R&D

Patients face many challenges in clinical trial settings

Enrolled **demographics are not representative of patient subgroups**, affecting trust, equity and medical efficacy

Our industry struggles to maintain R&D effectiveness amid evolving market dynamics

Only ~10% of agents investigated in the R&D phase **are developed and launched**

53% of launches have underperformed in the last 3 years while R&D spend across US pharma industry rose to **$83 billion**

Data sources in speaker notes section

Patient inclusive trials can address many of the challenges we face

Integrating representative patient needs in R&D can **enable more efficient recruiting** of different patient populations, decreased dropout, and **more meaningful endpoints**

Designing products and services to address unmet needs will **accelerate diagnosis**, drive earlier treatment starts, decrease never-starts, and **increase adherence**

Being an early mover in implementing **organization-wide patient Inclusivity** will expand partnership capabilities and **generate a strong goodwill** with industry stakeholders

patient partnership team

Health Authorities (HAs) across the globe are implementing Inclusive Research Guidances

The External Environment Is Aligning with Our Internal Patient Inclusivity Values

Regulatory Bodies Across the Globe

- **European Medicines Agency (EMA) - 2025 guidelines on inclusion of pregnant and breastfeeding women**
- **Diversity Action Plan (DAP) - The FDA rollout of the DAP: FDORA ACT 2022**
- **UK MHRA & HRA Guidance in development - Inclusion and Diversity Plan (IDP)**
- **Health Canada and the federal tri-agencies (CIHR, NSERC, and SSHRC) are actively working on Equity, Diversity, and Inclusion (EDI) Plan**

Our Framework for Impact

How Insights from Patient Communities Enhance Our Work

DISEASE & TREATMENT SOLUTIONS

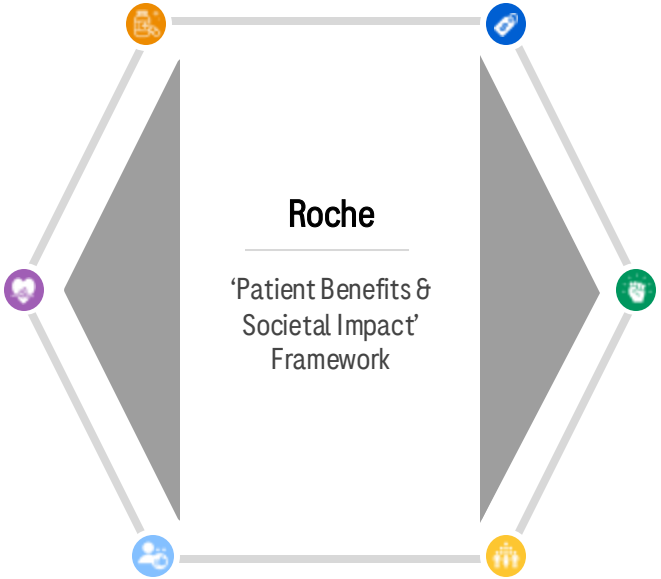
Improvements patients seek in their disease, the symptoms they experience, the treatments they use, and in their overall health

HEALTH RELATED QUALITY OF LIFE

Improvements patients & caregivers seek in how their disease and its treatments impact the physical, psychological and social aspects of their life

PATIENT PATHWAY

Improvements patients seek related to the steps and processes in the care pathway or across all stages of the patient journey (e.g. screening, diagnosis, monitoring, etc.)



FINANCIAL IMPACT

Improvements patients & caregivers seek in the direct and indirect cost of managing their disease (i.e., loss of income, out-of-pocket costs, cost of counseling, etc.)

PATIENT EMPOWERMENT

Improvements patients seek in their ability to engage in shared decision-making related to treatment choice and broader self-care options

SOCIETAL IMPACT

Improvements patients & caregivers seek in their local healthcare systems with a focus on access, investments in research, services that support patients, etc.

The 5 Principles (5Ps) Framework

5Ps can be considered a **conceptual structure** for **a measurable**, **transparent definition of clinical trial representativeness** that **can function within organizational constraints**.

The 5P framework was derived from simulating several studies in a pharmaceutical late-stage portfolio where components formed the basis for a data-informed framework to measure, standardize, and systematically define inclusive research.

https://link.springer.com/article/10.1007/s12325-025-03283-8

5Ps of Advancing Inclusive Research (AIR)

WHO?

Population Science Considerations (Biological/ Genetic/ Demographic)

1

WHERE?

Country & Site Selection to reach representative patient cohorts (Data-informed)

a. Geographical Proportionality Trial Enrollment (Region)

b. Accurate Demographic Representation (Country)

c. Representative real-world population (Site selection)

2

WHAT?

End-to-End Inclusive Trial Design (User and Data-informed)

3

WHO?

Patient-Reported Data Collection Standards (eCRF, SDOH, SOGI)

4

HOW?

Enabling Access to Trials

5

eCRF=electronic case report forms with self-reported race / ethnicity categories applicable globally; SDOH=social determinants of health; SOGI=sexual orientation and gender identity

PRINCIPLE 1

- **WHO? (Population)**
- WHERE?
- WHAT?
- WHO?
- HOW?

Population Science Data Considerations

WHO are patients with the disease?

Biological & Genetics Data:

- Biomarkers
- Ancestry
- Risk factors
- Others include:
  - Epidemiology
  - Disparities in diagnosis
  - Biology & progression of disease
  - ADME
  - Treatment patterns
  - Morbidity and mortality
  - Quality of care

Demographic Data:

- Race & Ethnicity
- Age & Sex

Roche Position on Race, Ethnicity, and Genetic Ancestry Application and Use in Clinical Trials and Product Development

Population Science descriptors (via literature reviews) translated and captured in HE&PS DAPA reports (#20) across Oncology, Neurology, Neuroimmunology, Ophthalmology, Respiratory, and Cardio-metabolic-renal disease

PRINCIPLE 2

WHO?

WHERE?

WHAT?

WHO?

HOW?

Country & Site Selection (Data-informed)

WHERE to reach representative patient cohorts

a. Geographical Proportionality Trial Enrollment (Region)

- Global trial enrollment by region is recommended in ICH17 guidelines on multiple regional clinical trials and should be proportional to the geographical burden of disease, as defined by appropriate reference metrics
- Data sources such as the US Cancer Statistics (USCS) for cancer in the US and the IHME GBD study globally, can be leveraged based on data quality, representativeness, and disease area coverage to provide epidemiology estimates at the indication or disease area level.

b. Enable Access and Commercialization (Country)

- Country-specific enrollment goals should adhere to standardized country-specific requirements and frameworks. Diversity outside the US cannot be captured or measured by US-specific demographic frameworks alone, such as the United States Office of Management and Budget's (US OMB) standards on race and ethnicity

c. Representative real-world population (Site)

- Data-informed opportunities to maximize inclusion that leverage real-world evidence and ensure clinical trials are representative of real-world patient populations. Data sources such as the US Census, USCS, SEER, EHR-derived real-world data (e.g., TrinetX, IHME, Flatiron), and genomic databases are available to obtain epidemiological estimates for US and global populations.
- Other HE&PS resources: AIR Calculator, R-index

Enrollment Goal Setting Guidance, DAP Template, AIR Calculator, R-Index

PRINCIPLE 3

WHO? (Population)

WHERE?

WHAT?

WHO?

HOW?

End-to-End Inclusive Trial Design

WHAT considerations to include in protocol design

Data-driven

Artificial intelligence algorithms combined with real-world data can potentially improve several aspects of clinical trials including:

- Patients screening
- Predict likelihood of patients who will enroll
- Extract features from electronic health records (EHRs)

ASCO evidence-based recommendations that laboratory tests should be used as exclusion criteria only when necessary. (link)

FDA has also developed a series of guidelines for eligibility criteria, however these are not standards and have not been widely implemented by many companies.

User Informed

Include input from providers, patient/patient advocacy groups, and investigators on the study trial design.

New PDG mandate: New protocols due to be finalised Q1 '24 onwards are developed with input and consideration for the patient

- Requirements for Patient Inclusivity: https://docs.google.com/document/d/1inWsD9BgYb1MpWF1Fb-QuKXrbvRgRust/edit?usp=sharing&ouid=109916580761344669801&rtpof=true&sd=true

Patient-Reported Data Collection Standards (eg. eCRF, SOGI) for Clinical Research

PRINCIPLE 4

- WHO? (Population)
- WHERE?
- WHAT?
- **WHO? (Patient)**
- HOW?

Current Roche eCRF Race or Ethnicity Categories	Proposed Roche eCRF Race or Ethnicity Categories	Race or Ethnicity eCRF Sub-categories	Directive No 15 (1997)	Directive No 15 (2024)
HISPANIC OR LATINO	HISPANIC OR LATINO	Mexican Puerto Rican Salvadoran Cuban Dominican Guatemalan Other Hispanic or Latino culture or origin	Hispanic or Latino	Hispanic or Latino
ALASKA NATIVE OR AMERICAN INDIAN	ALASKA NATIVE OR AMERICAN INDIAN	North American Indian Or Alaska Native Central American Indian South American Indian Other Indigenous Not Listed	American Indian or Alaska Native	American Indian or Alaska Native
ASIAN	ASIAN	Chinese Filipino Japanese Korean South Asian Vietnamese Other Asian Not Listed	Asian	Asian
BLACK	BLACK	African American African Caribbean Black African Other Black Identity Not Listed	Black or African American	Black or African American
INDIGENOUS AUSTRALIAN OR PACIFIC ISLANDER	INDIGENOUS AUSTRALIAN OR PACIFIC ISLANDER	Aboriginal Or Torres Strait Islander Maori Native Hawaiian Other Pacific Islander Not Listed	Native Hawaiian or Other Pacific Islander	Native Hawaiian or Other Pacific Islander
MIDDLE EASTERN, NORTH AFRICAN, OR WHITE	MIDDLE EASTERN OR NORTH AFRICAN	Middle Eastern North African	White	Middle Eastern or North African
	WHITE	White	White	White

Reconciliation of eCRF to FDA/OMB Race Categories

Optional SOGI Questionnaire for Clinical Research Survey

1. What is your sexual orientation?

a. Heterosexual
b. Lesbian
c. Gay
d. Bisexual
e. Queer
f. Questioning
g. Other, please specify
h. Prefer not to answer

2. What is your current gender identity?

a. Cisgender male
b. Cisgender female
c. Transgender male
d. Transgender female
e. Nonbinary
f. Other, please specify
g. Prefer not to answer

PRINCIPLE 4

WHO? (Population)

WHERE?

WHAT?

WHO? (Patient)

HOW?

Patient-Reported Data Collection Standards (cont. SDOH Questionnaire) for Clinical Research

Education

What is your level of education/schooling?

[ ] Elementary School
[ ] Middle School
[ ] Some High School
[ ] Graduated from High School
[ ] Some College
[ ] Graduated from College
[ ] Post-Graduate

Employment Status

Before your diagnosis, what was your employment status?

[ ] Full-time Employed
[ ] Part-time Employed
[ ] Self-employed (either full-time or part-time)
[ ] Retired
[ ] Not employed

Health Insurance Status

What type of health insurance do you have, if any. Please select one:

[ ] Private/Commercial
[ ] Medicare
[ ] Medicaid
[ ] Veteran Affairs (VA) Health Plan
[ ] Other
[ ] I do not have a health insurance

Income Status

What is your annual household income?

[ ] E< $15,000
[ ] $15,000 - $34,999
[ ] $35,000 - $49,999
[ ] $50,000 - $74,999
[ ] $75,000- $99,999
[ ] $100,000 - $149,999
[ ] >$150,000

Marital Status

What is your marital status?

[ ] Single (never married)
[ ] Married (including common law)
[ ] Separated
[ ] Divorced
[ ] Widowed
[ ] Domestic Partnership (same sex, opposite sex or unregistered)

Zip Code of Home Residence

What is the zip code of your home residence?

Maximize Patient Access to trials

Enhanced Patient Support Services (EPSS)

Pre-Enrollment

- **Patient community assessment/support**
  - Establish baseline level of support: comfort with **technology**, study requirements, workplace and **childcare needs**, transportation
  - **Identify support groups, patient advocacy groups**
- **Financial Assessment/Support**
- Transportation Support
- **Translation** Support
- **Family Information and Follow Up**
- **Patient Tour** and Intake Support

On-Study Support

- **Active Visit Support**
- Follow Up Support Call

Study Completion / Follow-up

Study completion, feedback collection about trial participation

- **Patient integration to treating Physician after study completion**
- Sharing of trial information/results

- A well defined catalogue of invoiceable services
- Built into study budget for consistency and sustainability
- Sites determine who is best suited to provide each service
- Rates vary depending on seniority of role performing the service (~2,000 - ~3,000 USD per patient)

Principle 1
Biological/genetic and population science considerations

***Who* is the population most affected by multiple myeloma?**

Patient demographics

Race	
White	74%
Black	21%
Asian/Pacific Islander	3%
American Indian/Alaska Native	0.7%
Ethnicity	
Non-Hispanic	91%
Hispanic	9%
Age	
<40	1%
40-64	34%
≥65	65%
Sex at birth	
Male	55%
Female	45%

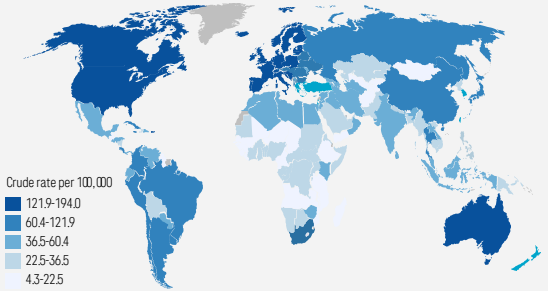
Patient risk factors

Race	2-3x higher incidence in African Americas than other race/ethnic origins
Gender	1.5x higher in men than women
Age	Older age of diagnosis, median age of diagnosis 69 years old Younger median age of diagnosis among Black patients compared to White patients
Family history	2-4x risk 1st degree relative with MM
Environmental exposures	Post 9/11 workers, exposure to agent orange in Vietnam War, pesticides and chemicals

Principle 2
Data-driven country and site selection

***Where* are the patients most affected by multiple myeloma?**

Incidence per 100,000 people



Region	Number of cases	%
Africa	6882	4
Americas	52,190	28
United States	4779	3
East Mediterranean	7309	4
Europe	53,889	29
Southeast Asia	23,117	12
Western Pacific	44,541	24

Principle 3
User informed end-to-end inclusive trial design

***What* will the multiple myeloma trial design look like?**

- Broaden inclusion and exclusion criteria for late-stage studies
- Optimize assessments for patients and healthcare system (eg, blood volumes, visits)
- Co-creation with the Clinical Trials Expert Council

Principle 4
Patient-reported demographic identification

***Who* are the patients participating?**

- Standardized eCRF, SDOH, SOGI (optional, self-reported)
- Race/ethnicity collection with county and region categorizations; subject to regulation limitations
- Report on OMB categories at analytic stage

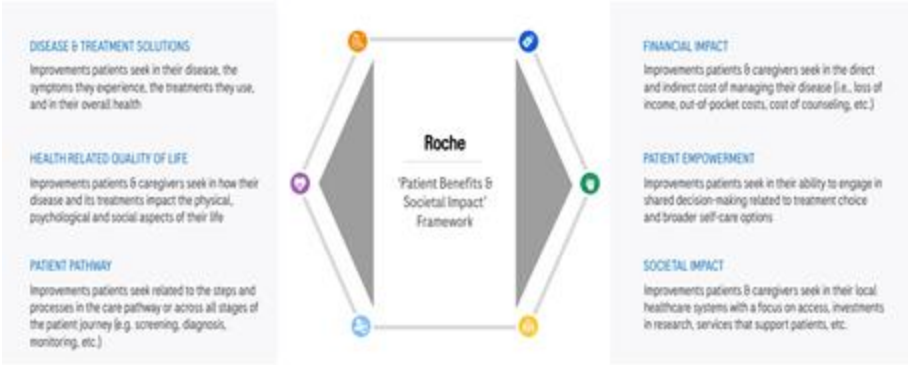
Principle 5
Enabling access to trials

***How* will patients access trials and treatment?**

- Patient-centric clinical trial elements
- Standardized Enhanced Patient Support Services
- Conduct trials in countries where there is a sustainable path to regulatory and access approval

Bringing together Representative Patient Insights & Population Health Data

PBSI Framework



5P AIR Framework

- Shaping More Inclusive & Relevant Clinical Trials
- Enabling More Robust & Scientifically Valid Data
- Driving Equitable Market Access & Adoption by building payor and regulator confidence

Population Health Data (The "What") + Patient Insights (The "Why") = Inclusive Strategy (The "How")

Evolution from traditional silos to holistic data sets

	Previous Approach - Fragmented Data		Current Approach - Inclusive & Robust Data
FOUNDATION	Trial convenience and often in healthy, homogenous populations		Equitable Outcomes: Scientifically mirroring the real-world disease burden
DATA	Historical trial data and standard epidemiological statistics	with	Merging Population Science (epidemiology/SDoH) direct Patient insights (qualitative insight). CHIMES (MS)
SITE SELECTION	“Tried and true" academic centers; highly localized to research hubs.	»	High-burden geographic areas and community centers identified by population data. EMPACTA (COVID-19)
DATA QUALITY	Representative of a selected subgroup; potential biology gaps and generalization issues		Captured across a full spectrum of genetic ancestry, socioeconomic realities, and comorbidities. ELEVATUM (Ophtha)
END-TO-END and	Primarily focused on safety efficacy endpoints, and geographic limitations		Data informs early disease strategy, clinical design, operational planning, and global access ELEVATUM (HbA1c)