

Is the Older Adult Subgroup of the Rare Disease Population Marginalized on Purpose? An Analysis of Equity Issues Encountered By the (OLDER) Rare Disease Population

Jean Pierre Uwitonze<sup>1,2</sup>, Lize Duminy<sup>1,2</sup>, Daniel Ammann<sup>3</sup>, Carl Rudolf Blankart<sup>1,2</sup>

- 1) KPM Center of Public Management, University of Bern, Bern, Switzerland
- 2) Swiss Institute for Translational and Entrepreneurial Medicine, sitem-insel, Bern, Switzerland
- 3) Institute for Health Policy and Health Economics, Bern University of Applied Sciences, Bern, Switzerland

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Background

Rare disease populations face hurdles throughout their interaction with health systems. These hurdles often vary in most aspects of rare diseases. We deduce that these hurdles could subject the rare disease population to differential treatment in comparison to the general population. Consequently, the rare disease population might experience different forms of equity issues.

Objectives

- To find evidence of equity issues experienced by rare disease populations including the older adult subgroup of the general rare disease population:
1. Identify equity issues experienced by rare disease populations in comparison to the general population.
  2. Identify equity issues experienced by older adults living with a rare disease in comparison to the general rare disease population.

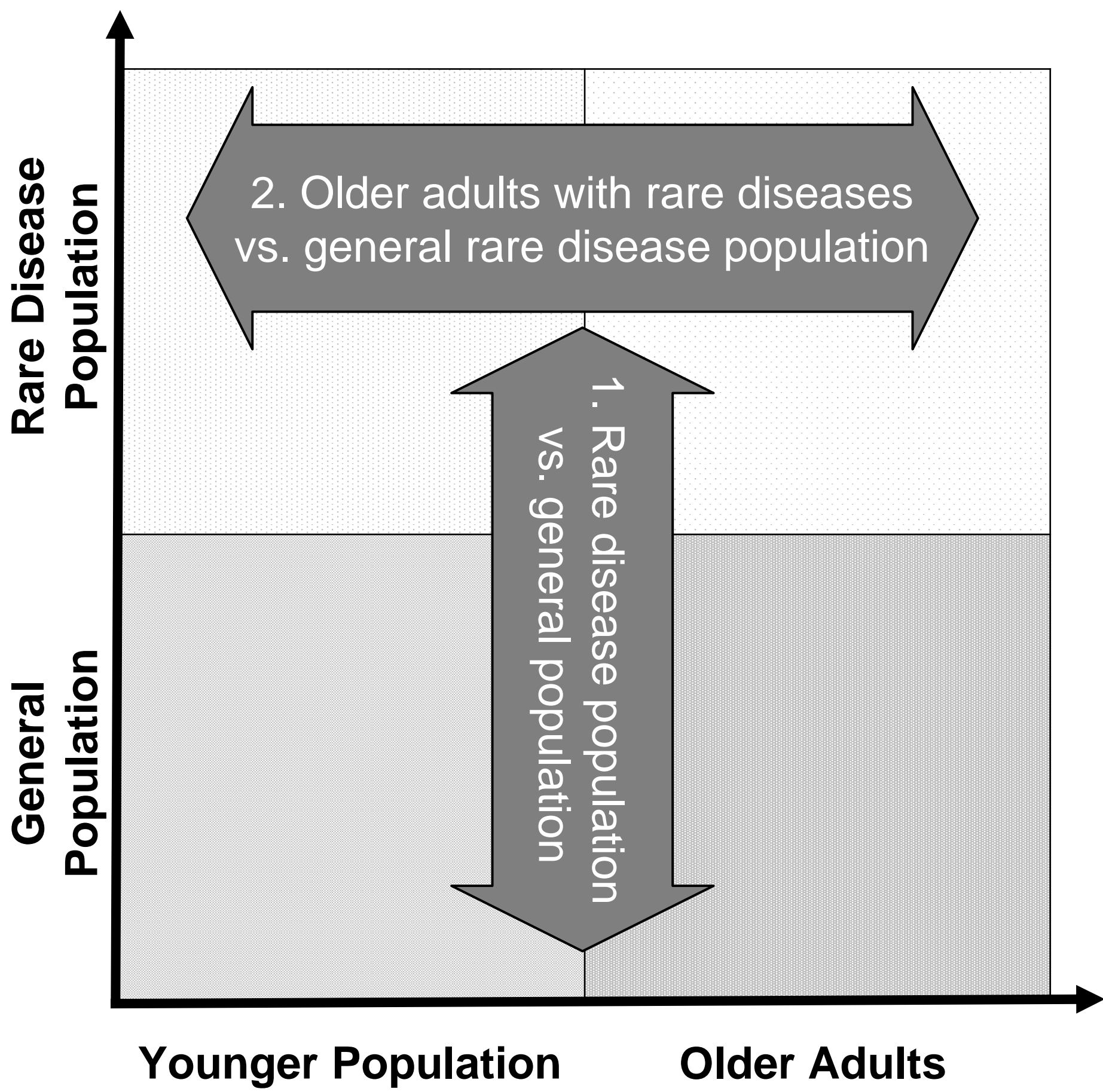


Figure 1: Illustration of the different dimensions of population and comparison groups that may experience equity issues.

Methods

**Systematic literature review** according to the Preferred Reporting Items for Systematic Reviews and Meta-Analyses (PRISMA) guidelines. Search strategy based on search terms related to **equity** and **rare diseases**. *MEDLINE via PubMed, the Cochrane Library, and Embase* were searched. Consideration of gray literature of Regulatory bodies, patient organizations, industry, and others.

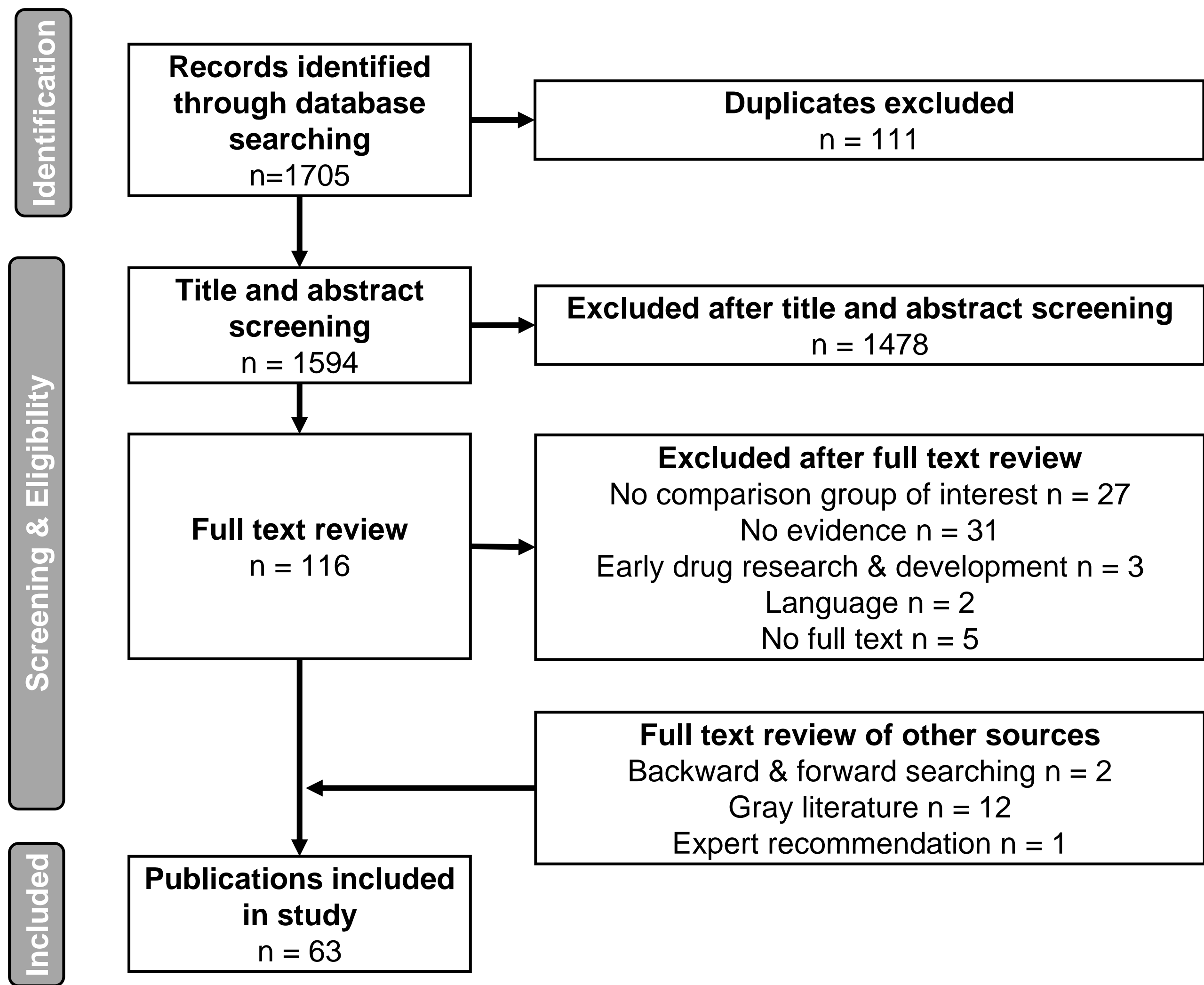


Figure 2: PRISMA flow chart shows chronology of our publication selection process.

- Extraction of evidence by two reviewers.  
Inductive evidence clustering.  
Development of Ethical Spectrum and Implementation Framework with:
- 2 main evidence clusters
  - 5 evidence subclusters

Results: Ethical Spectrum and Resource Allocation Framework

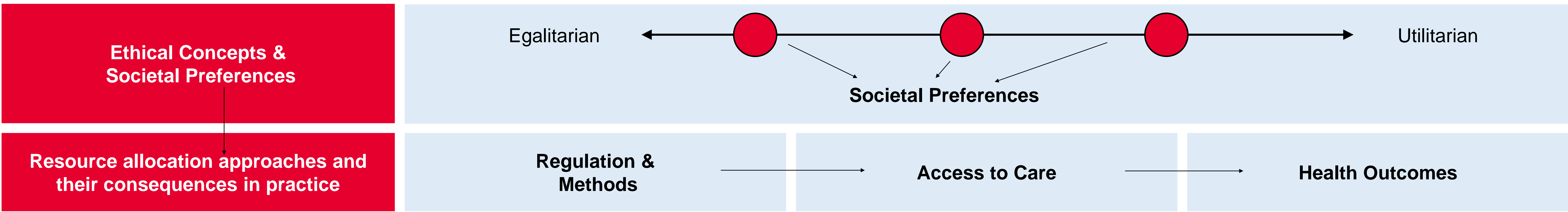


Figure 2: Inductively developed Ethical Spectrum and Resource Allocation Framework showing areas where equity issues may be encountered. From the moral principles and **ethical concepts** that countries and their respective health systems choose to align with based on their values, **societal preferences** emerge. As a result, health policies, **regulations and methods** are put in place to guarantee the population’s **access to care**, thus influencing **health outcomes**.

Discussion

**Ethical concepts:** Despite each society having a unique historical and philosophical perspective on the distribution of resources, the main schools of thought regarding resource allocation and health policy can be situated along a continuum of the two most common objectives of resource allocation i.e., egalitarianism and utilitarianism.

**Societal preferences:** There is little to no preference for rarity compared to common diseases.

**Regulations & methods:** Generally less favorable towards rare disease treatments.

**Access to care & health outcomes:** Evidence of lower primary care access and poorer age adjusted mortality rates for older adults with rare diseases. However, evidence base remains limited and insufficient.

**Ageism:** Evidence gap of ageism within specific population subgroups e.g., rare disease patients.

Take Home Message

We could not find evidence that demonstrates equity issues among the older rare disease population in comparison to the general rare disease population. Rare disease populations encounter comparatively more equity issues in their care trajectory compared to the general population. We call for further research exploring the age dimension of equity issues experienced within specific subgroups of the general population. The Ethical Spectrum and Implementation Framework can be used as a starting point in science, policy or public affairs.