

Current outcome  
measures in Haemophilia  
relevance to patients

---

# Content

---

Health economic perspective

- What are outcomes?

Patient perspective

- What are outcomes

So what?

Comments

# Health economic perspective

---

- Health related quality of life (HRQoL)
- Morbidity
- Mortality
- Annualised bleed rate (ABR)
- Target joints
- Cost
- Joint scores
- Bone mineral density

# Health economic perspective

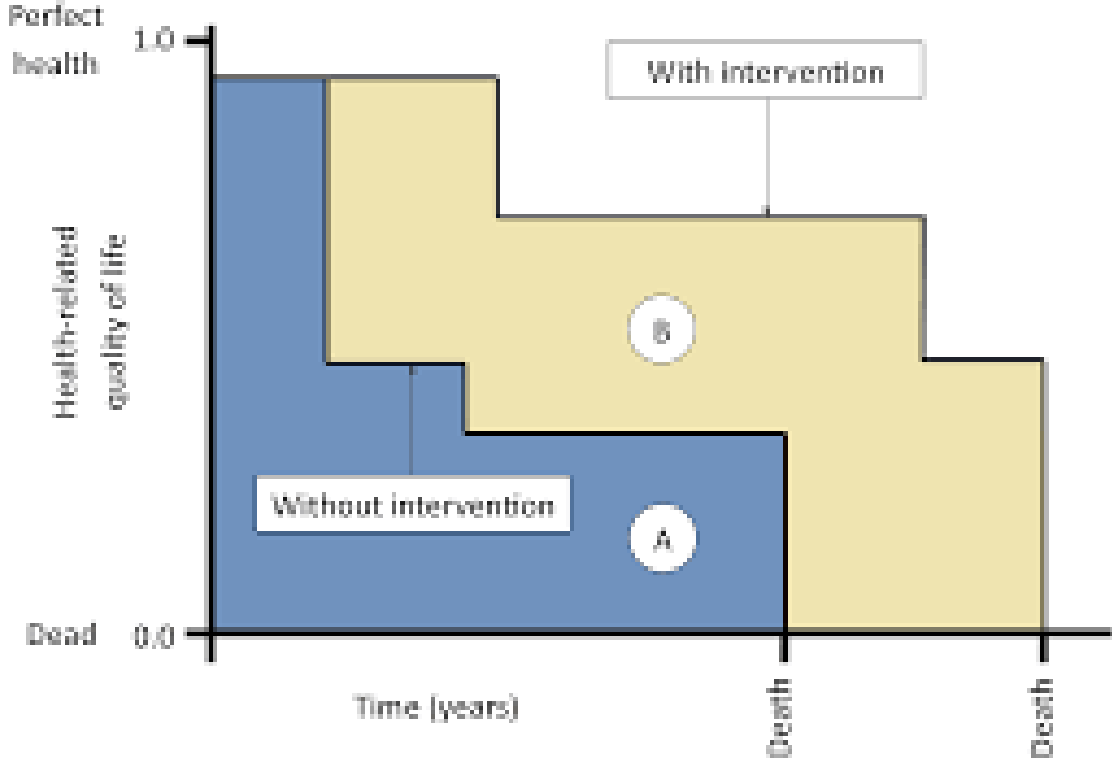
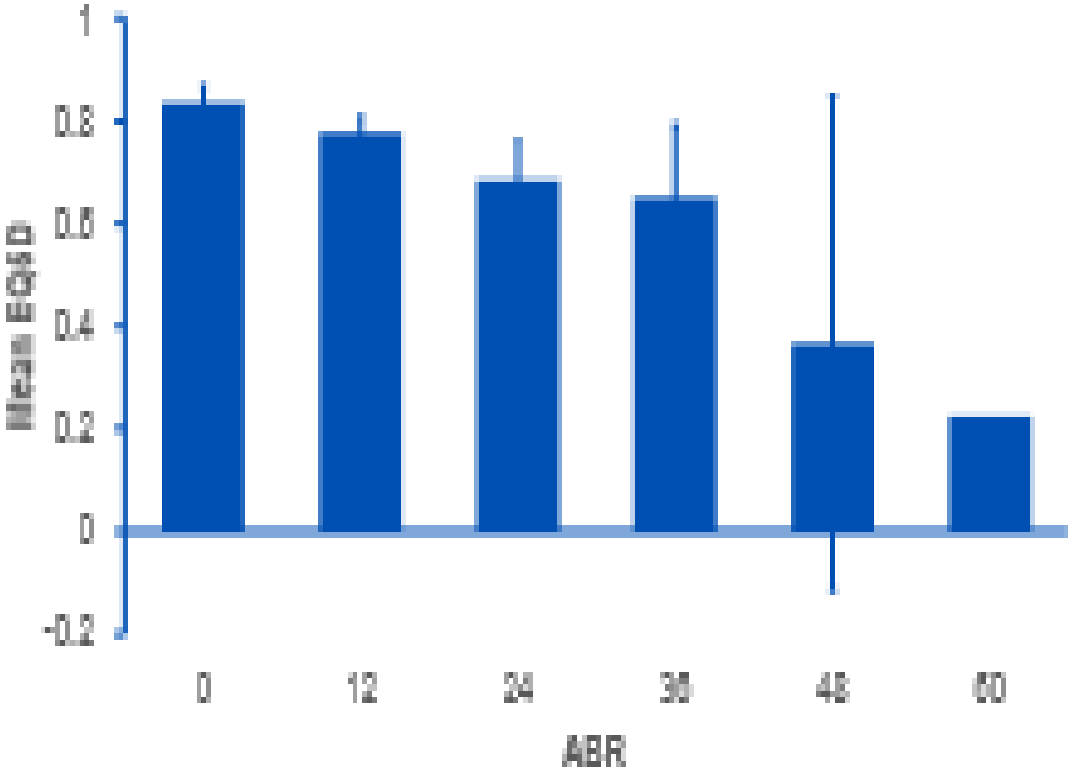
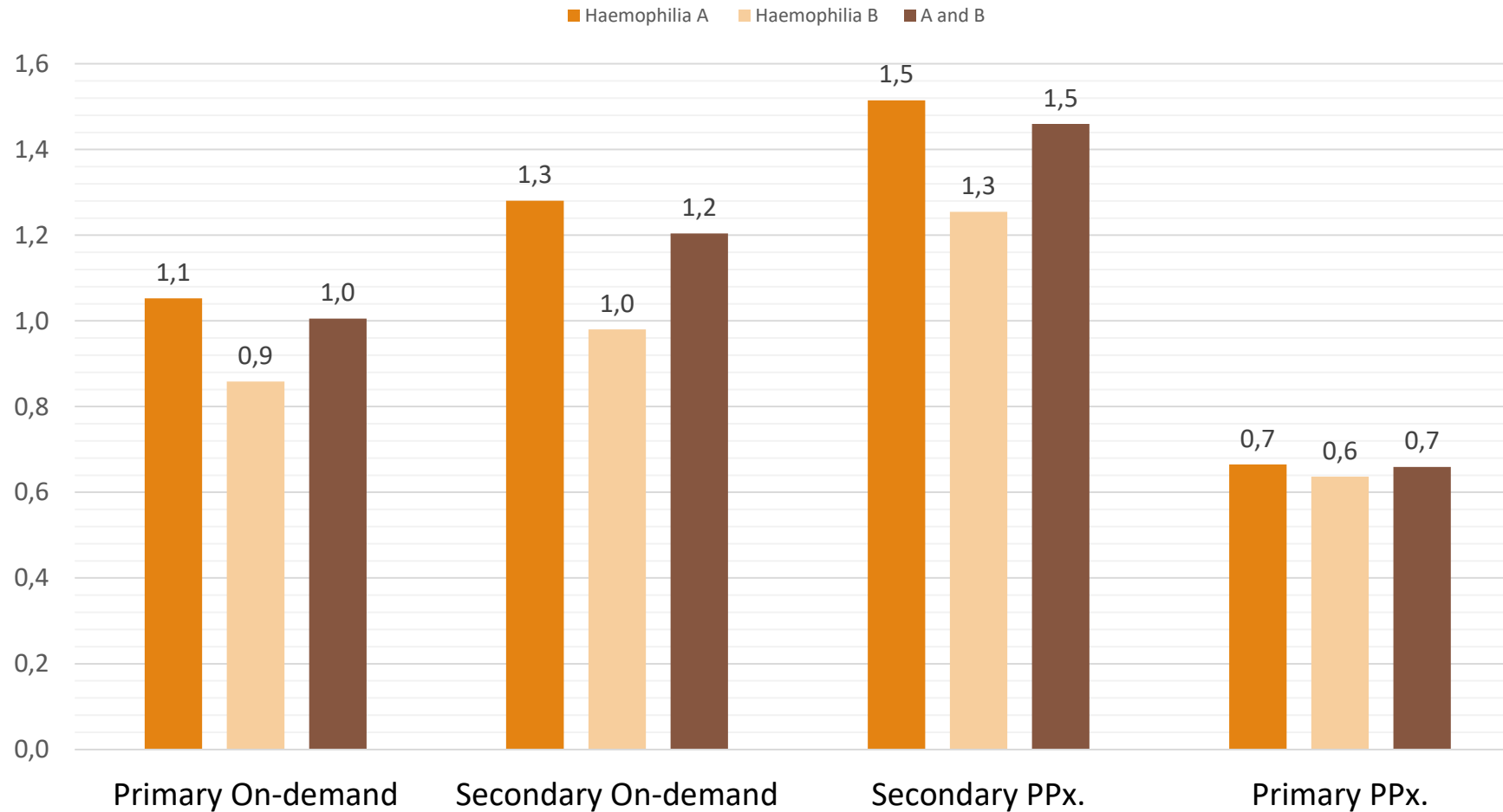


Figure 3: EQ-5D by ABR



# Target joint incidence by treatment type...

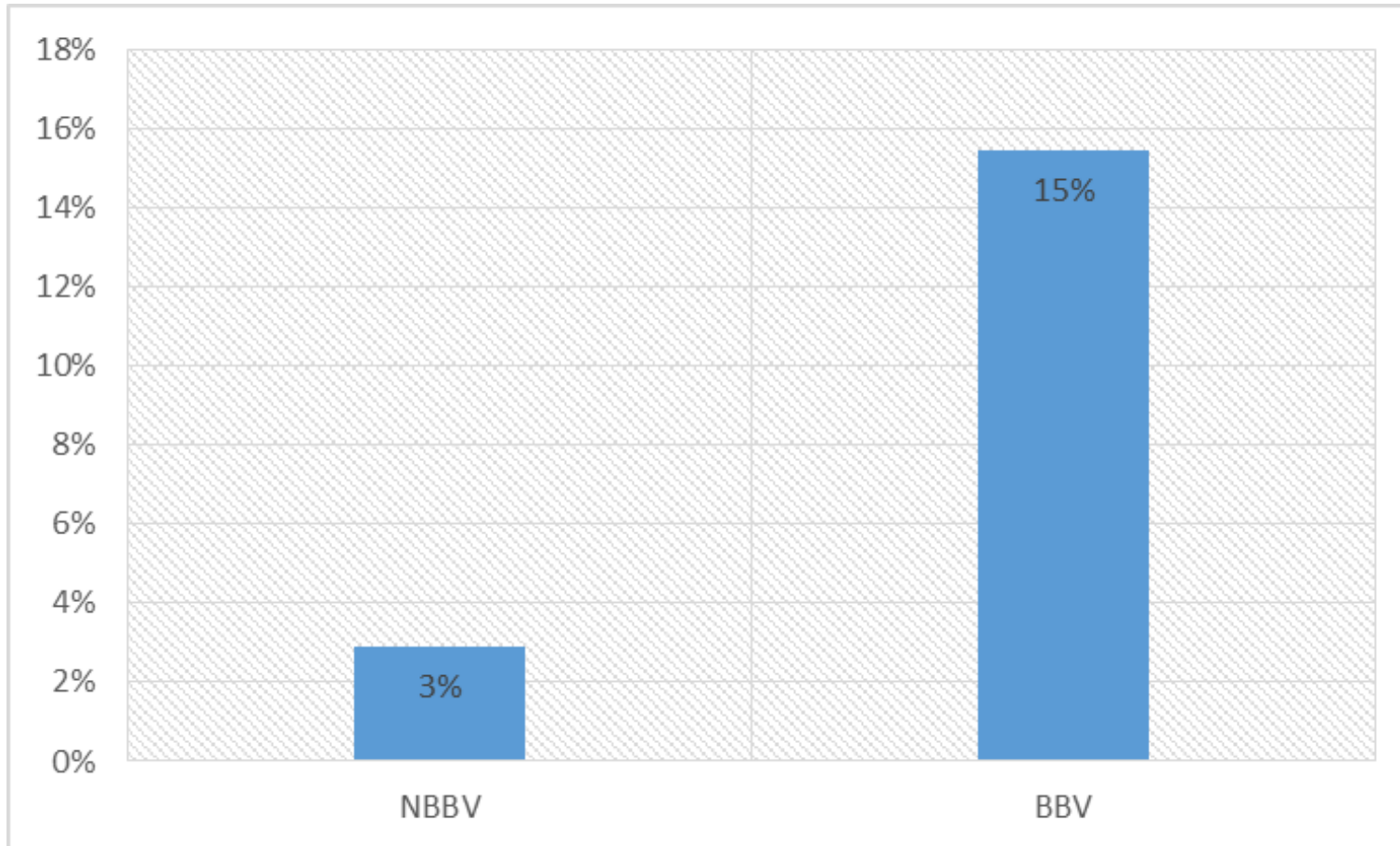


# Patient perspective

---

- Same as above?
- Workloss
- Caregiver burden
- Number and location of target joints
- Etc

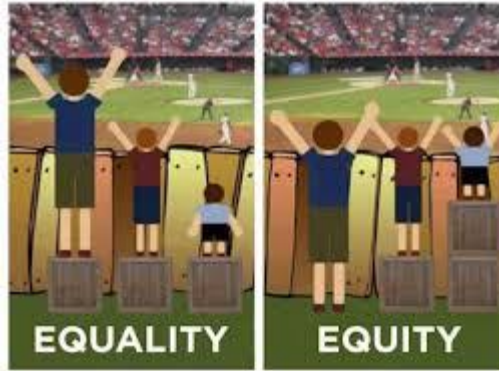
# Impaired Ability To Work



Illustrated is the proportion of each cohort that is unable to work due to health reasons. The results show the sample population of BBV patients unable to work as 5 times higher than the NBBV population.

# So what...

---





# Comments

---

- Resources are finite so there need to be criteria to distribute available resources efficiently (degree of pragmatism needed)
- Until we consider a more holistic/disease specific outcomes it will be difficult to ensure 'fairness' in the way we distribute our resources
  - Due to this, the severity/impact of some, particularly rare, diseases are not properly considered (market failure important here too)
- Fixed process based health economic evaluation is largely inappropriate in rare diseases.
- Apparent heterogeneity of patient characteristics/outcomes