

THE PERSPECTIVE OF THE PATIENT ON UPSTREAM CONVERGENCE AND DOWNSTREAM DIVERGENCE

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EUPATI FELLOWS

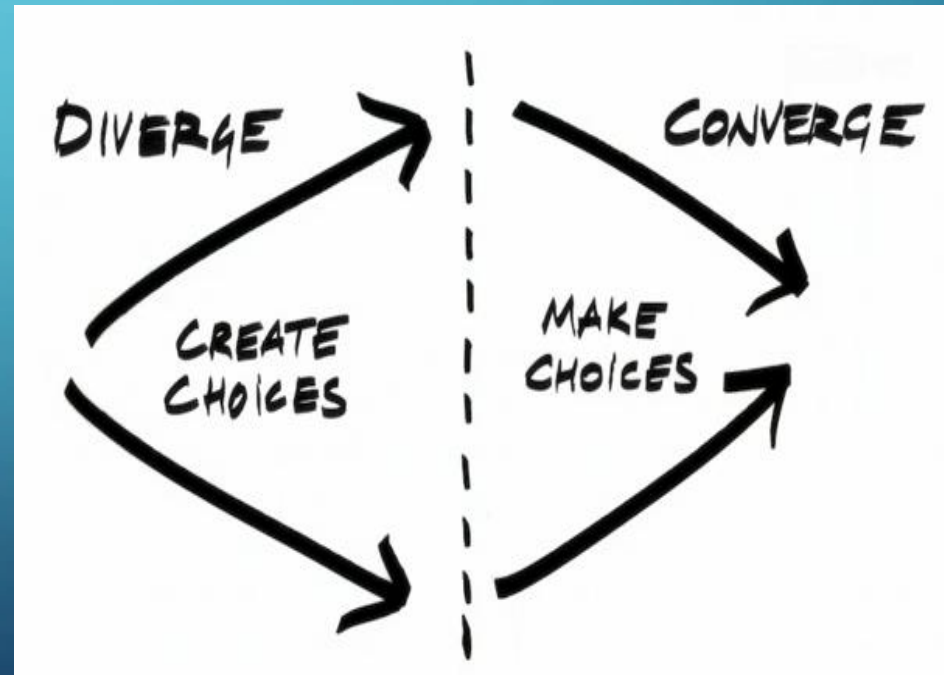
NVN PATIENT ADVOCATES

MEMBER OF THE RSNN ADVISORY BOARD



THE PATIENTS' PERSPECTIVE

REGULATORY FAST APPROVAL



UNEQUAL ACCESS TO MEDICINE AND TREATMENT AFTER APPROVAL BY EC DUE TO NATIONAL DIFFERENCES IN REGULATION IN EUROPE

Europe

- Differences in access
- Health benefits

The Netherlands

- Approved bij European Commission
- Patient perspective on access

A PATIENTS' PERSPECTIVE REIMBURSEMENT INSTEAD OF REGULATORY

Focus on costs:

Availability differs across different countries:

- Prescription at the front door, deliverance via back door/ PO box medicine
- Medicine shortage / delayed access to medicine
- Costs of medicine (generic vs brand medicines)
- Medicine tourism
- Access to expensive medicine/ orphan drugs



PATIENTS' PERSPECTIVE: WHAT CAN WE DO?

- **Infrastructure:**

Patient organisations; patients can easily stand up for their interests and cooperate with each other and stakeholders thanks to well organised patients' organisations.

- **Mobility/ technology:**

Access to a computer/ internet is not common for every patient. Sharing such technology has impact on privacy and may cause an extra threshold to stand up for your rights.

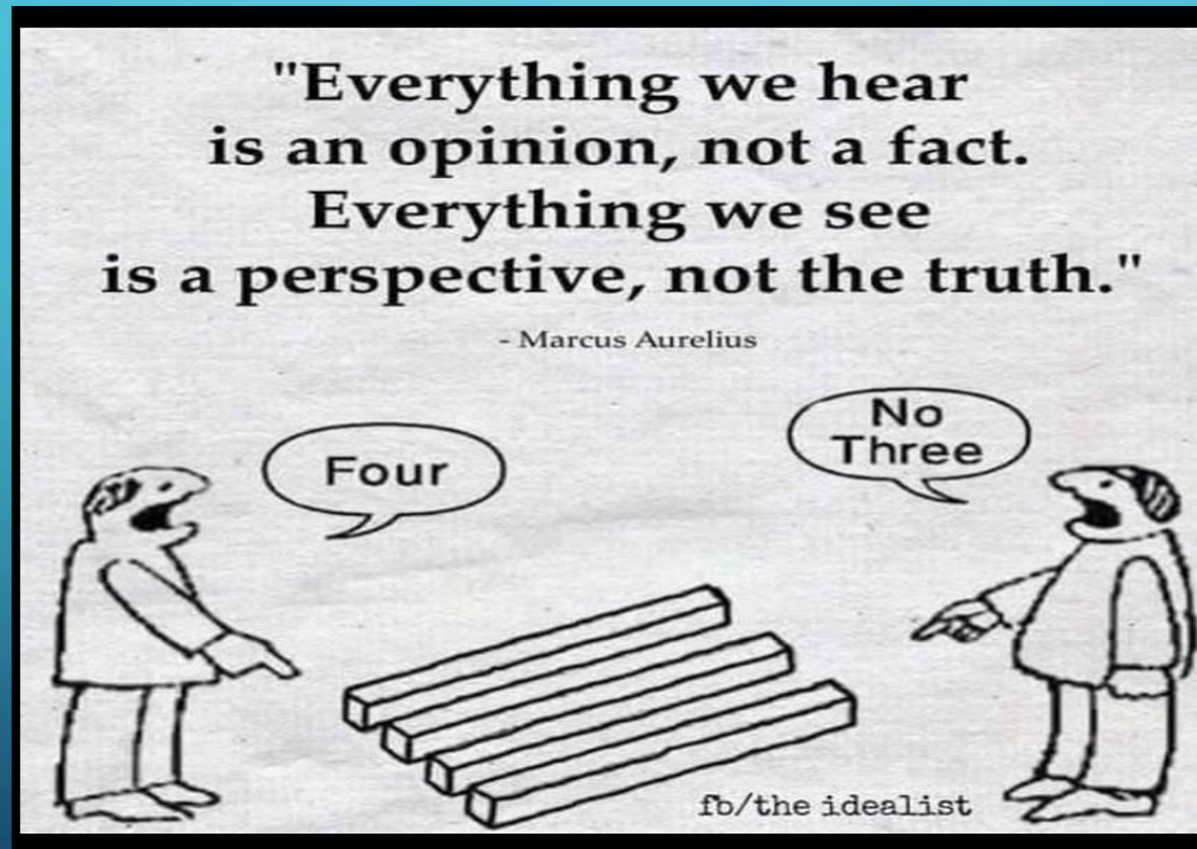
Financial resources to be able to travel abroad for access to a medicine or a treatment



EUPATI: Patient Engagement Through Education
Providing patients with accessible and reliable information and training on medicines research and development and other therapeutic innovations. A patient-led partnership working across stakeholders to enhance patient engagement.

**HOW TO REACH THE RIGHT PARTNERS AS A
Patient ADVOCATE?**

A PATIENTS' PERSPECTIVE



ATYPICAL HUS

- Orphan disease, estimated incidence in the Netherlands
15-20 cases a year (5 children, 10-15 adults)
- Disorder (genetic) in the complementsystem
- Relapsing, therefore Chronic character
- Kidney failure
- Diagnosis is difficult

ATYICAL HUS TREATMENT

- Plasma exchange , the cornerstone of treatment.
- Promising new drug: Eculizumab.
- Treatment protocol : lifelong , every two weeks
- Expensive : €500.000 a year per patient

THE PATIENT IN ACTION

- Research : the company, patients abroad
- Expertcentre: vision on a different protocol, cooperation between academic centres, working on a Dutch guideline



CURIEHUS

- Support from Zorgverzekeraars Nederland and ZonMW
- Start and stopcriteria, personalized treatment
- Steering committee with patient involvement
- Business case
- Cost effectiveness

MINISTRY OF HEALTH

- Reimbursement during the four years of the study
 - Treatment using the Dutch guideline
 - Both Patient and Doctor support the registry
- Price negotiations with the Company
- Orphan drug arrangement

THE RESULT OF DIVERGENCE

- **On the positive side:**

- a lot of research is done based on CureiHus
- The company has accepted the Dutch approach
- The study reduced the costs of the medicine significantly

- **On the negative side:**

- approximately 80% of the aHus patients in the world have no access to the correct cure