

# Cystinosis: Adherence to cysteamine treatment in Benelux

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# Context

- Orphan metabolic chronic disease
- Treatment: **Cysteamine (Cystagon<sup>®</sup>)**
  - better medium & long term prognosis
  - Every 6 hours
  - Side effects: bad breath & smell, gastro-intestinal
  - Orders received at Orphan Europe (OE)
- Monitoring: WBC-cystine levels
- **Follow Up forms sent to OE (4/year):**
  - Weight, Height, GFR
  - Cystagon<sup>®</sup> prescribed dosage
  - Cystine levels
  - Estimated adherence to treatment

# Objectives

## Primary

Evaluate adherence to Cystagon<sup>®</sup> treatment in Benelux in 2006

## Secondary

- Compare Belgium & The Netherlands
- Determine at risk groups
- Correlation between cystine levels & “Cystagon<sup>®</sup> adherence ratio”

# Method: our postulate !

Adherence : concordance between Cystagon®

- prescribed by the physician
- ordered by the pharmacist to Orphan Europe (consumption)

Pharmacy Stamp	Age 65yrs (ethn) 216/1597	Sex, Forename, Surname & Address SMITH John 22 Bridge Street Anytown KB1 5SK
<p>Prescriber's Signature and Date</p> <p>Number of days allowed Nil (more than a month)</p> <p>Indications: Dimorphine 50mg ampoules Supply 6(six) ampoules 60mg daily by subcutaneous infusion over 24 hours (No more items on this prescription)</p>		
<p>Signature: <i>D.O. Good</i></p> <p>Date: 02/03/06</p>		
<p>For Supply to: Dr D O Good 345543 7 High Street Anytown KB1 CD2 Tel: 0111 222 333</p>		
<p>NHS FPI0NC0103</p>		



# “Cystagon adherence ratio”

$$\frac{\text{Yearly (Orders received - prescribed dosage)}}{\text{Yearly prescribed dosage}}$$

**LOW RATIO: < - 0.2 (< - 20%)**

Examples:

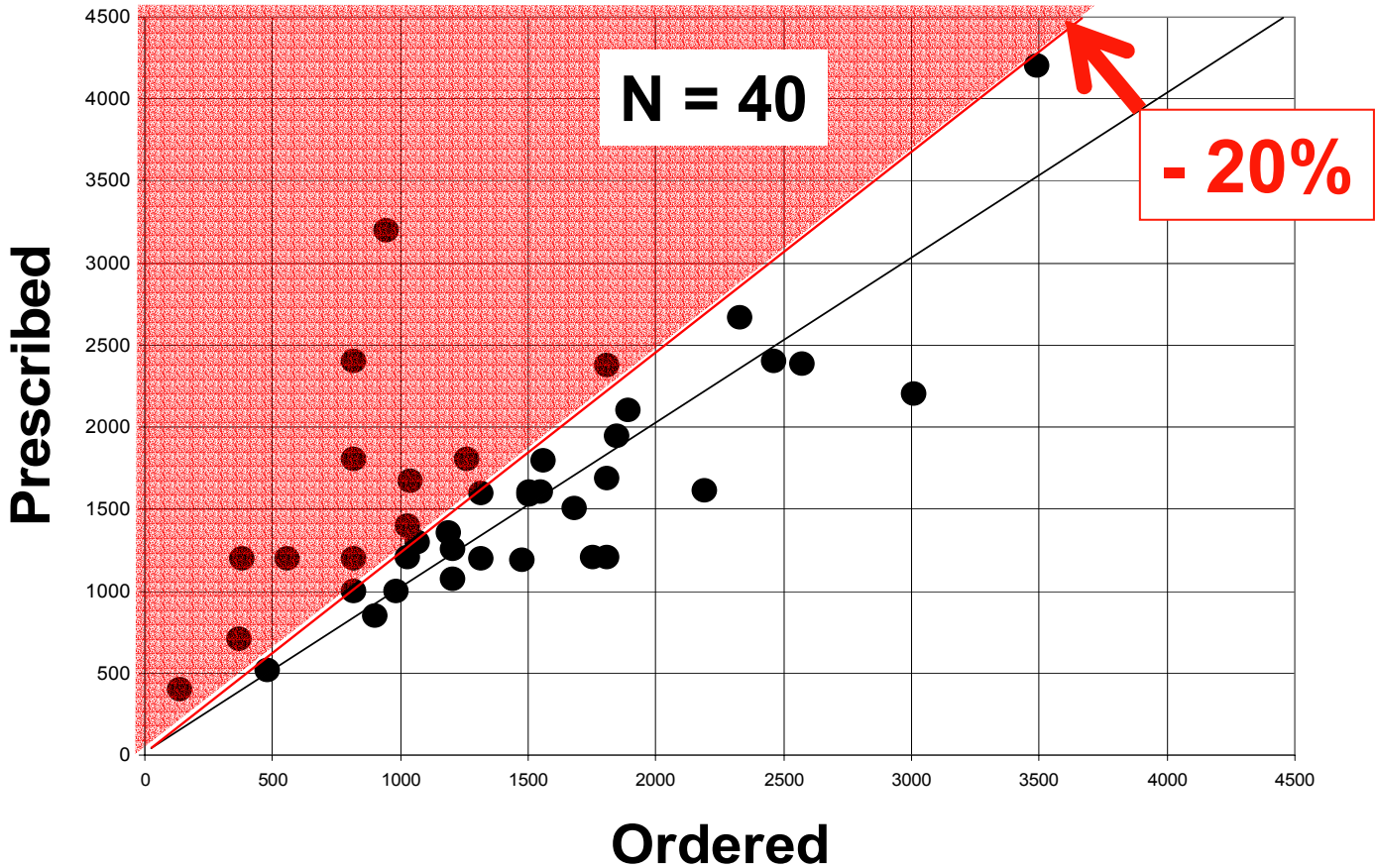
$(50\text{g} - 80\text{g}) / 80 = - 0.38 (- 38\%)$  Low “adherence rate”

# Population

- **Inclusion criteria**
  - Cystagon<sup>®</sup> in Benelux on the whole year 2006
- **Exclusion criteria**
  - Missing data on Follow up forms: **13%**
- **40 Patients included: 30 NL & 10 BE**
  - Age : 3 – 43 y.o. (6 > 21 y.o., median 13)
  - Genders: 20 F & 20 M

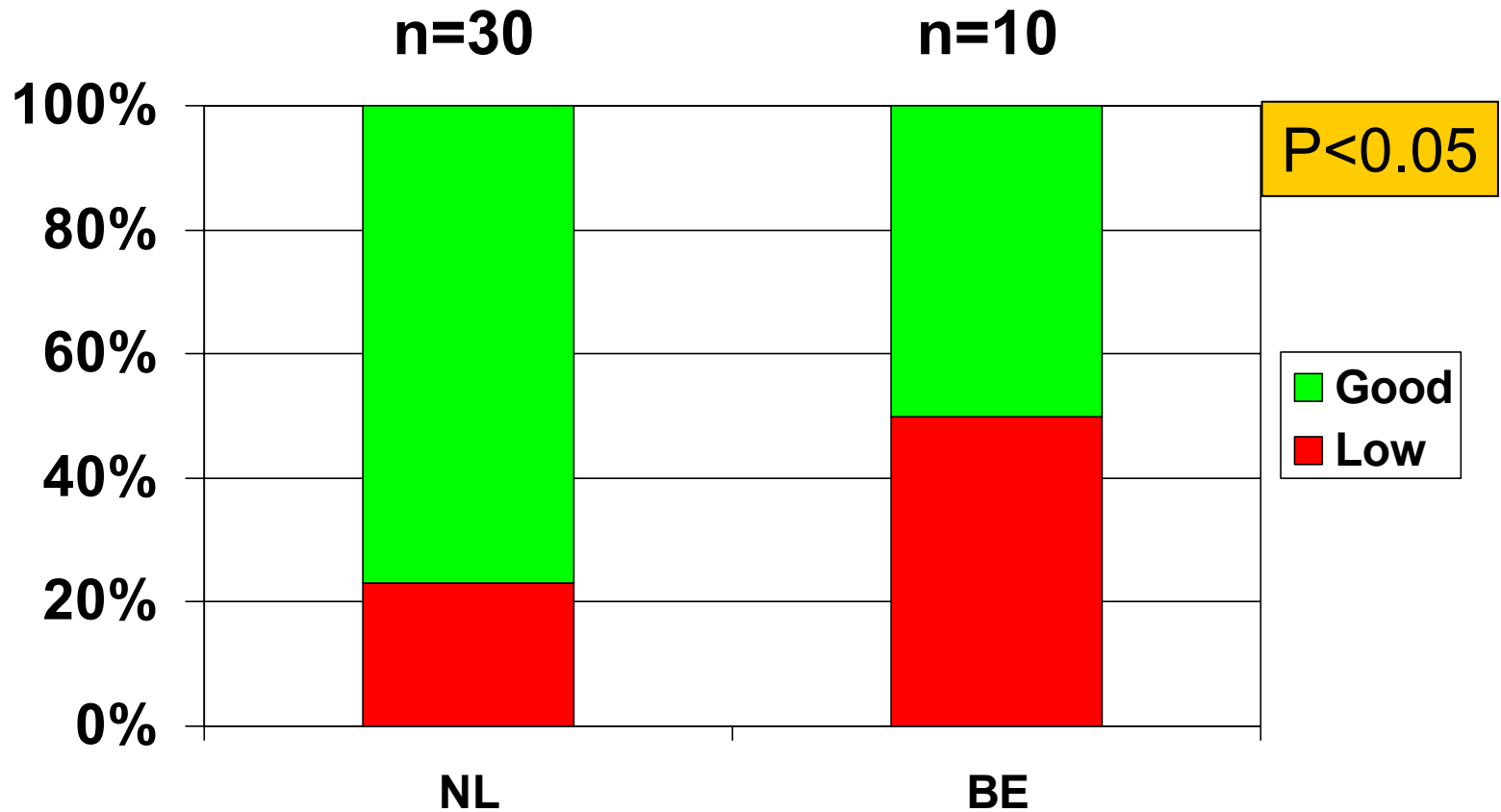
**NB: Prescribed daily dosages = Guidelines (97%)**

# Cystagon adherence ratio" 2006



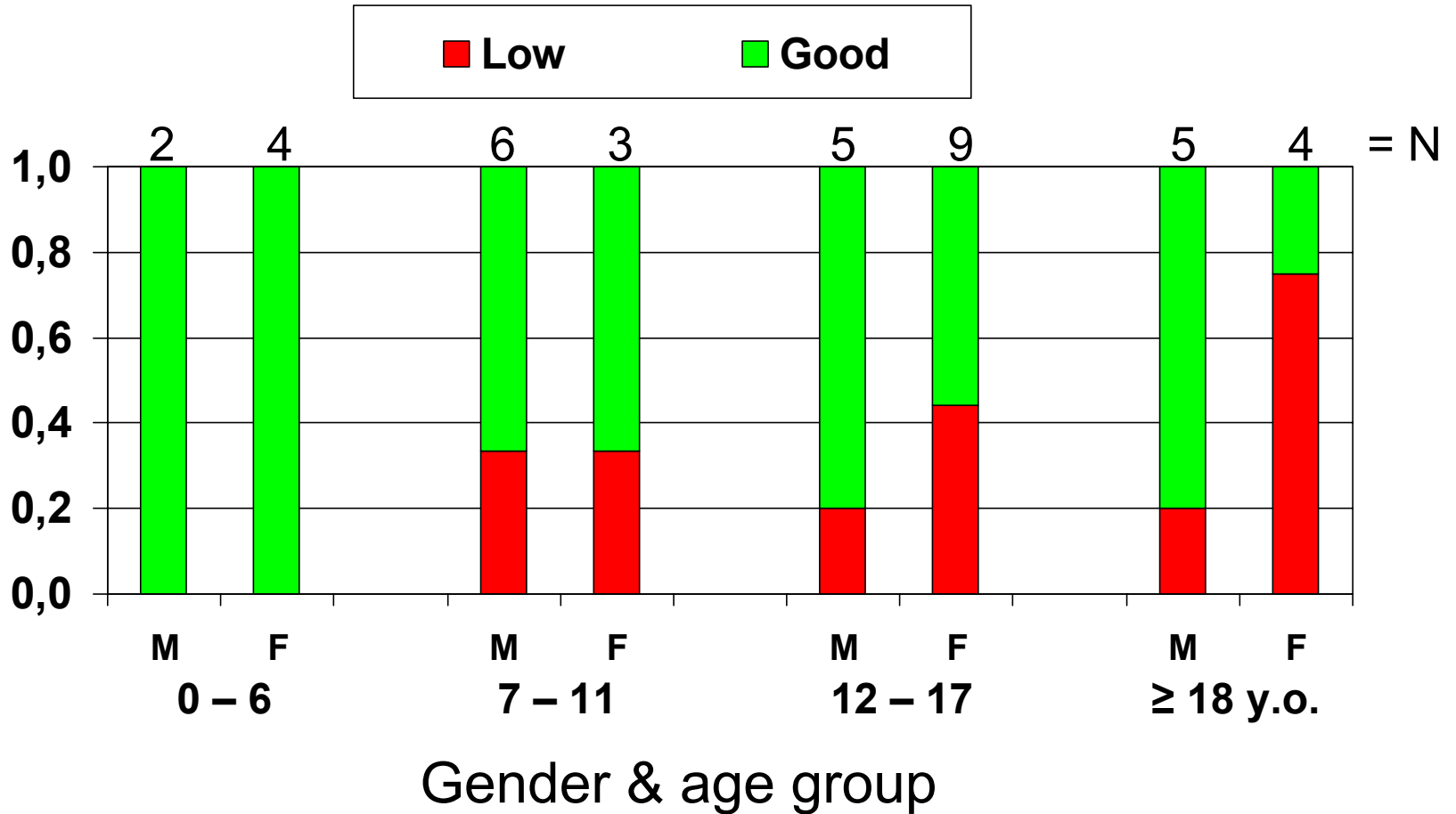
**Low ratio: 30% of patients**

# “Adherence ratio” by country



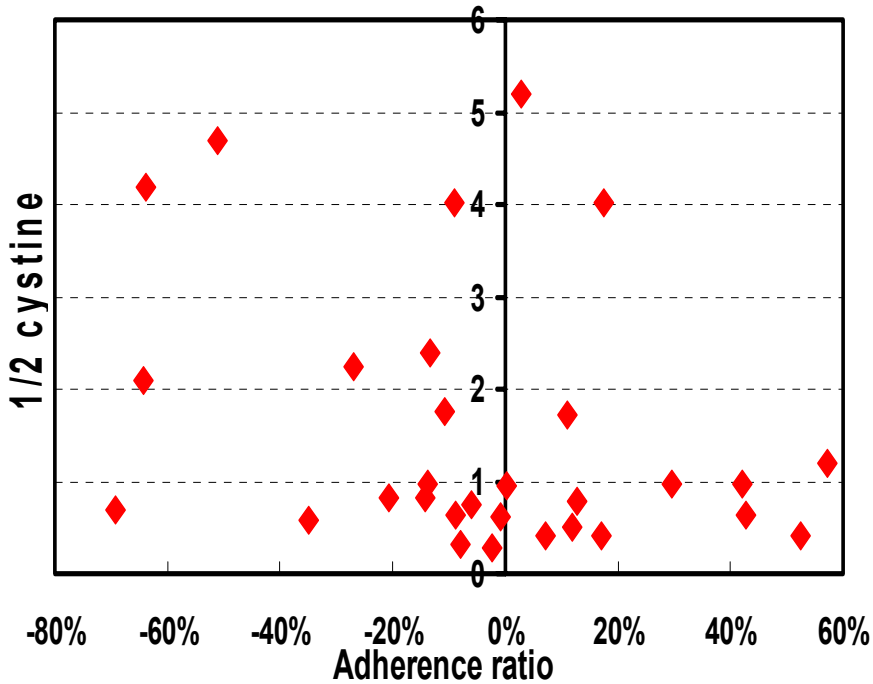


# “Adherence ratio” by gender & age group

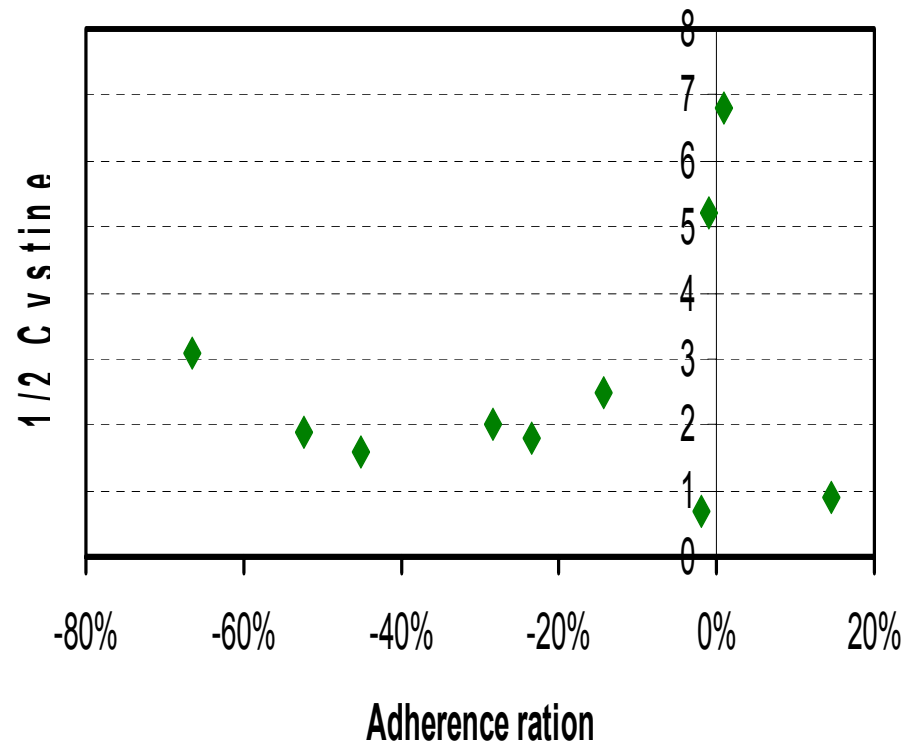


# Cystine levels and “adherence ratio”

The Netherlands



Belgium



# Discussion : “Adherence ratio”

- **Reliability?**
- **Dutch > Belgian ?**
  - Cystagon<sup>®</sup>: NL 50mg & 150mg vs BE 50 mg
  - Patients’ centralisation: NL > BE
  - ≠ organisation: delivery? stocking?
- **↘ adolescent girls & women?**
  - < bad breath & smell?
- **+ Age 3-6?**
  - < parents supervision?

# Conclusions: it suggests

- “Low Cystagon adherence ratio”: 30% of patients
- At risk population
  1. Belgian?
  2. Adolescent girls & women?
- “Cystagon adherence ratio”  $\Leftrightarrow$  Cystine levels
- We need:
  - to improve adherence & adherence indicators
  - to standardize cystine levels measurements
  - International quality controlled database of patients