

# 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	Sex, Forename, Surname & Address
	65yrs (male)	SMITH John 216/1997 Anytown KB1 5SK
Please see 'Adherence' tab for Number of days assessed N.B. Please see method		
Indications	Dimorphine 50mg ampoules Supply 6(six) ampoules 60mg daily by subcutaneous infusion over 24 hours (No more items on this prescription)	
<b>SAMPLE</b>		
Signature	Date	
<i>D.O. Good</i>	02/03/06	
For Supply to	Anyborough Health Authority	
Dr D O Good 345543	7 High Street	
Anytown KB1 CD2	Tel: 0111 222 333	
<input type="checkbox"/>		
NHS	FP10NC0103	



# “Cystagon adherence ratio”

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

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

Examples:

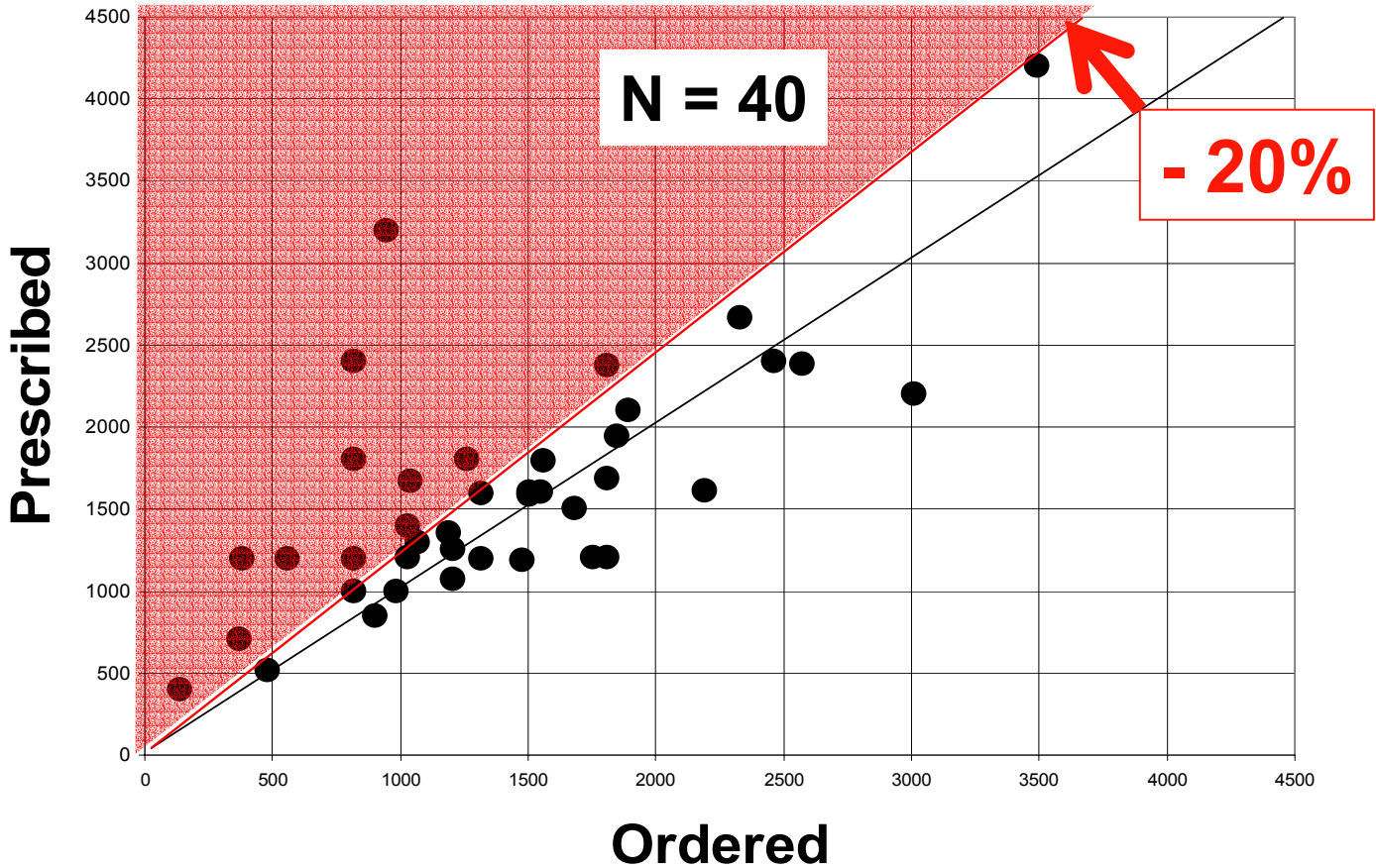
$(50g - 80g) / 80 = - 0.38 (- 38\%)$  Low “adherence rate”

# 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

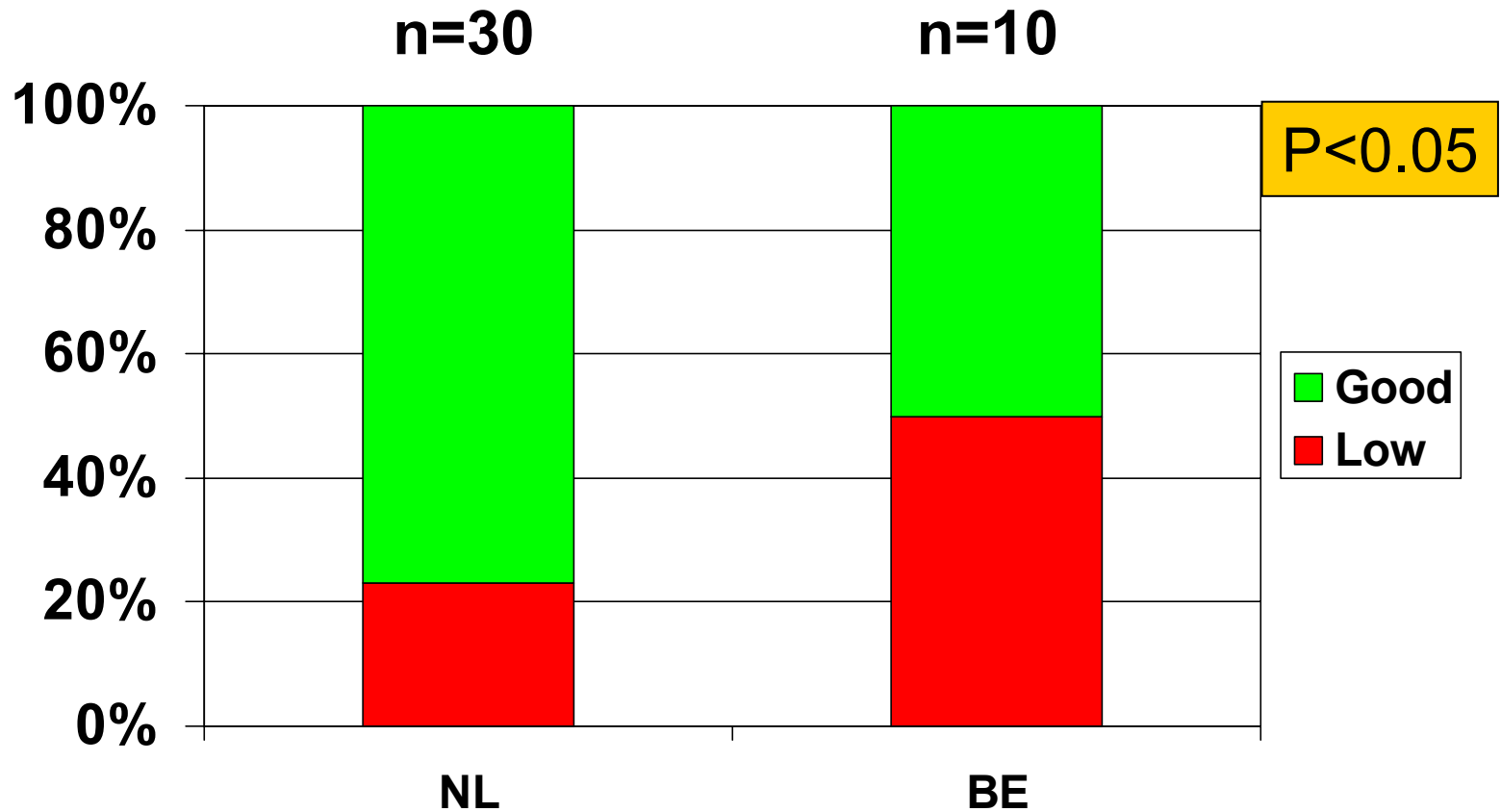
**NB: Prescribed daily dosages = Guidelines (97%)**

# Cystagon adherence ratio" 2006



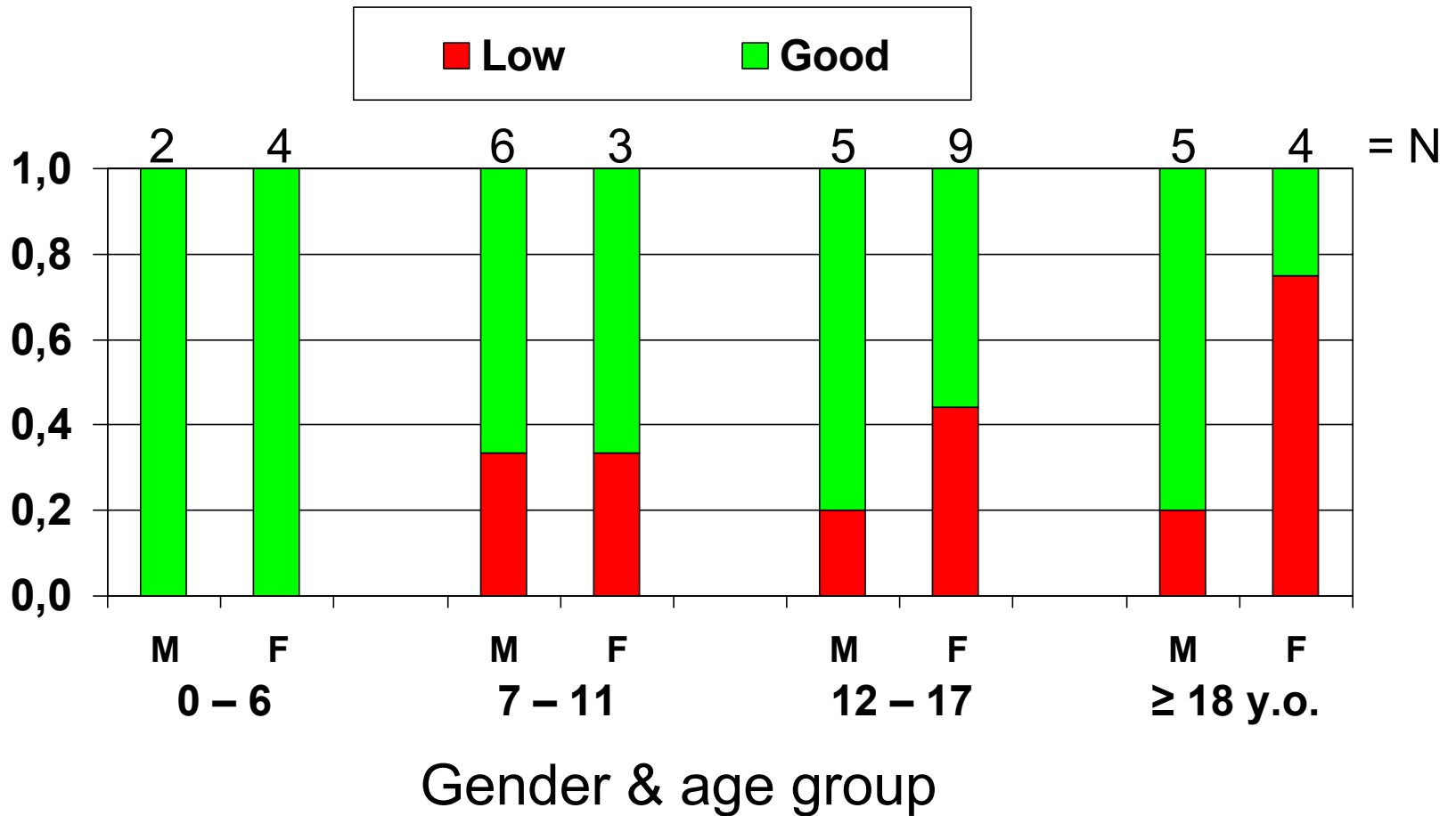
**Low ratio: 30% of patients**

# “Adherence ratio” by country



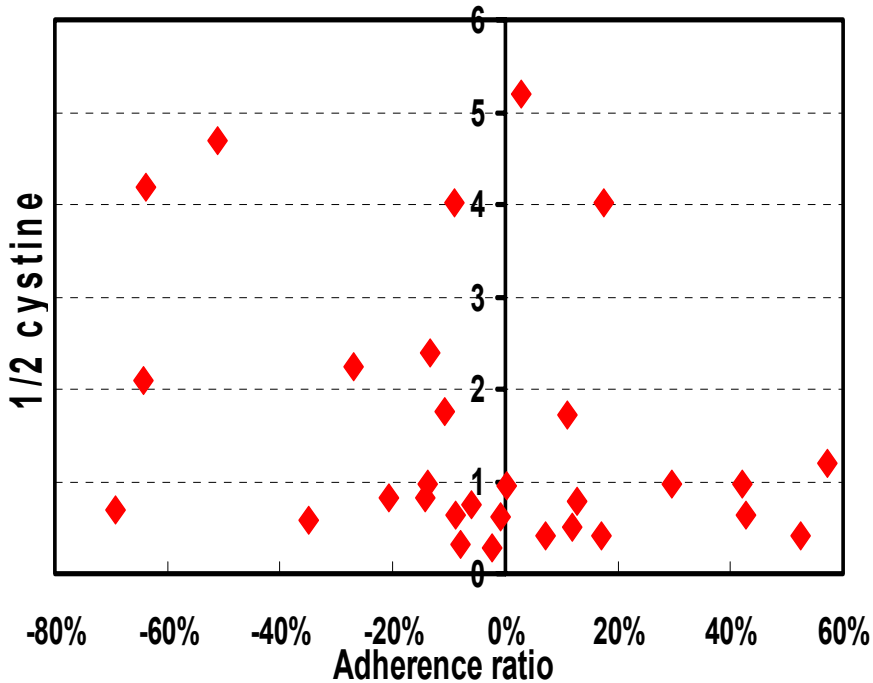


# “Adherence ratio” by gender & age group

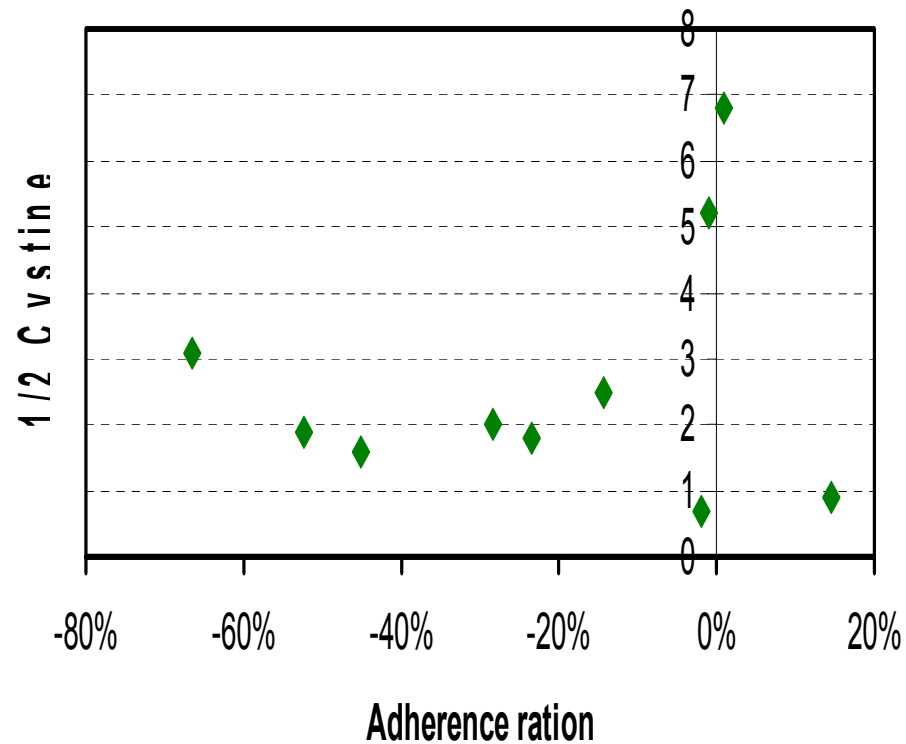


# Cystine levels and “adherence ratio”

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