

# The Cystinosis Patient Central: A Survey to gain insight into the medication use and the perspective for treatment improvement from Dutch Cystinosis Patients

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## Patient Questionnaire

- Medication use
- Main side effects of cystinosis treatment
- Patient-oriented perspective on treatment improvements

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## Results

### Demographics

17 Dutch cystinosis patients

|                     | ≥ 18 years (n=10)  | < 18 years (n=7)   |
|---------------------|--------------------|--------------------|
| Sex                 | 4 female<br>6 male | 5 female<br>2 male |
| Age (years)         | Range 18-41        | Range 2-12         |
| Transplantation (n) | 6                  | None               |
| Dialysis (n)        | 1                  | None               |
| Tube (n)            | None               | 4                  |
| Capsules per day    | 46 (23.5-86)       | 34 (range 6-63)    |

## Conclusion

**High number of capsules per day**

**There is an unmet patients need for treatment improvements**

**Find new strategies to alleviate the problems faced by patients:**

- Make relatively accessible and affordable adaptations to the medicinal treatment of cystinosis
- New (public or public-private) partnerships should be pushed for delivering these improvements on a cost-based and timely manner to patients who could benefit significantly

### Main side effects

Upset stomach (7/17)

Unpleasant body and breath odor (7/17)

Nausea (7/17)

Blurred vision / eye irritation and pain after using eye drops (8/17)

### Patients Needs

Reducing dosing frequency (11/17)

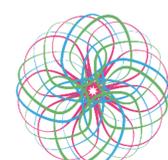
Reducing the number of capsules per day and size (11/17)

Finding improvements or alternatives for the eye drops (11/17)



### Medication use

See overview below



**CHDR**  
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# Overview Daily medication use Dutch Cystinosis Patients

## NO TRANSPLANTATION (n=11)

| Medication                                | Patient population (n)     | Total number of capsules/tablets per day | Dosing moments per day | Route of administration |
|---|----------------------------|--|------------------------|-------------------------|
| <b>Cystine Depleting Therapy**</b>        | <b>11</b>                  |  |                        |                         |
| Cystagon®                                 | < 18 years (n=3)           | 14 (range 6-24)                          | 4                      | oral                    |
|   | ≥ 18 years (n=2)           | 20                                       | 4                      | oral                    |
| Procysbi®                                 | < 18 years (n=4)           | 20 (range 12-27)                         | 2* (range 2-3)         | oral                    |
|   | ≥ 18 years (n=2)           | 24 (range 16-32)                         | 2                      | oral                    |
| <b>Treatment Corneal Cystine Crystals</b> | <b>11</b>                  |  |                        |                         |
| Cystadrops®                               | < 18 years (n=7)           | 3 drops in each eye (range 1-4)          | 3 (range 1-4)          | eyes                    |
|   | ≥ 18 years (n=4)           | 4 drops in each eye (range 2-6)          | 4 (range 2-6)          | eyes                    |
| <b>&lt; 18 years</b>                      | <b>&lt; 18 years (n=7)</b> |  |                        |                         |
| Growth hormone treatment                  | 5                          | 1 ampul                                  | 1                      | subcutaneous injection  |
| Vitamin D                                 | 6                          |  |                        |                         |
| Vitamin D                                 | 4                          | 1.5 (range 1-3)                          | 1.25 (range 1-2)       | oral                    |
| Vitamin D (solution)                      | 3                          | 9 drops (range 4-10)                     | 1                      | oral                    |
| Phosphate                                 | 5                          |  |                        |                         |
| Phosphate                                 | 3                          | 8 (range 4-12)                           | 4                      | oral                    |
| Phosphate (solution)                      | 2                          | 42.9 ml (range 8.8-60 ml)                | 4 (range 3-4)          | tube                    |
| Potassium                                 | 7                          |  |                        |                         |
| Potassium                                 | 5                          | 10 (range 3-20)                          | 3 (range 3-4)          | oral                    |
| Potassium (solution)                      | 5                          | 68.4 ml (range 40-140 ml)                | 4 (range 3-4)          | tube                    |
| Sodium Bicarbonate                        | 3                          |  |                        |                         |
| Sodium Bicarbonate                        | 1                          | 15                                       | 3                      | oral                    |
| Sodium Bicarbonate (solution)             | 2                          | 44 ml (range 28-60 mg)                   | 4                      | tube                    |
| <b>Gastrointestinal complaints</b>        | <b>3</b>                   |  |                        |                         |
| Acid reflux                               | 2                          | 2.5 (range 1-4)                          | 1.5 (range 1-2)        | oral                    |
| Acid reflux (solution)                    | 1                          | 7.5 ml                                   | 1                      | tube                    |
| Hypertension                              | 2                          | 1.25 (range 0.5-2)                       | 1.5 (range 1-2)        | oral                    |
| <b>Others</b>                             | <b>6</b>                   |  |                        |                         |
| Metabolism                                | 2                          | 2 (range 1-2.5)                          | 1                      | oral                    |
| Metabolism (solution)                     | 2                          | 3.9 ml (range 3-4.8 ml)                  | 3.5 (range 3-4)        | tube                    |
| Laxative (solution)                       | 2                          | 2 sachets (range 1-3)                    | 1                      | oral + tube             |
| Anti-inflammatory                         | 1                          | 2  | 2                      | oral + tube             |
| Antibiotic                                | 1                          | 1  | 1                      | oral                    |
| <b>≥ 18 years</b>                         | <b>≥ 18 years (n=4)</b>    |  |                        |                         |
| Vitamin D                                 | 2                          |  |                        |                         |
| Vitamin D                                 | 1                          | 2  | 1                      | oral                    |
| Vitamin D (solution)                      | 1                          | 3 drops                                  | 1                      | oral                    |
| Phosphate                                 | 2                          |  |                        |                         |
| Phosphate (solution)                      | 2                          | 73 ml (range 56-90 ml)                   | 3.5 (range 3-4)        | oral                    |
| Potassium                                 | 3                          |  |                        |                         |
| Potassium                                 | 3                          | 15 (range 12-42)                         | 3                      | oral                    |
| Potassium (solution)                      | 1                          | 56 ml                                    | 4                      | oral                    |
| Calcium Carbonate                         | 1                          | 18                                       | 3                      | oral                    |
| Acid reflux                               | 2                          | 1  | 1                      | oral                    |
| Hypertension                              | 2                          | 1  | 1                      | oral                    |
| <b>Others</b>                             | <b>4</b>                   |  |                        |                         |
| Metabolism                                | 3                          | 3 (range 2-4)                            | 3 (range 1-3)          | oral                    |
| Laxative (solution)                       | 1                          | 1 sachet                                 | 1                      | oral                    |
| Heart failure                             | 1                          | 4  | 2                      | oral                    |
| Immunosuppressant                         | 1                          | 9  | 2                      | oral                    |
| <b>WITH TRANSPLANTATION (n=6)</b>         |                            |  |                        |                         |
| <b>Cystine Depleting Therapy**</b>        | <b>6</b>                   |  |                        |                         |
| Cystagon®                                 | > 18 years (n=1)           | 12                                       | 3                      | oral                    |
| Procysbi®                                 | > 18 years (n=5)           | 22 (range 16-38)                         | 2 (range 2-3)          | oral                    |
| <b>Treatment Corneal Cystine Crystals</b> | <b>5</b>                   |  |                        |                         |
| Cystadrops®                               | > 18 years (n=5)           | 3 drops (range 2-9)                      | 3 (range 2-3)          | eyes                    |
| <b>Transplantation therapy</b>            | <b>6</b>                   |  |                        |                         |
| Vitamin D                                 | 5                          | 1 (range 1-2)                            | 1                      | oral                    |
| Potassium                                 | 2                          | 19 (range 1-37)                          | 2 (range 1-3)          | oral                    |
| Magnesium Hydrate                         | 1                          | 4  | 2                      | oral                    |
| Iron deficiency                           | 2                          | 1.5 (range 1-2)                          | 1.5 (range 1-2)        | oral                    |
| Acid reflux                               | 4                          | 1 (range 1-2)                            | 1 (range 1-2)          | oral                    |
| High cholesterol                          | 3                          | 1 (range 0.5-1)                          | 1                      | oral                    |
| Hypertension                              | 4                          | 1.5 (range 0.5-7)                        | 1.5 (range 1-3)        | oral                    |
| <b>Others</b>                             | <b>2</b>                   |  |                        |                         |
| Anticoagulant                             | 1                          | 1  | 1                      | oral                    |
| Metabolism                                | 1                          | 2  | 2                      | oral                    |
| Intestinal cramps                         | 1                          | 1  | 1                      | oral                    |
| Pain and infection                        | 1                          | 3.5 (range 1-6)                          | 2 (range 1-3)          | oral                    |

\*OR drops, ml or number of sachets/ampuls per day.

\*\*Since the introduction of Procysbi® (2021) in The Netherlands, 9 out of 15 patients previously on Cystagon® treatment made a switch to Procysbi®, 3 patients remained on their Cystagon® treatment and for 2 patients this information was not available.

– Non-daily medication is not presented –

