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Health related Quality of Life in patients with inherited unconjugated hyperbilirubinemia (Crigler-Najjar syndrome): a multicentre study.

Background/aims:

Development of Novel treatment strategies, including gene therapy, evaluation of the HRQL in to evaluate the potential beneficial effects of the novel treatment strategies.

Study design/Methods or/PROMS:

- Baseline study in all and in candidates before and after treatment paper-pencil-survey, Castor database
- PROMS: General questions, SF-34 in adults and the PredsQL in children

Target population:

Patients with Crigler-Najjar syndrome and their family. Aim to include > 50 patients

Languages:

Dutch, German, French, Italian

Countries:

AmsterdamUMC and Erasmus MC (The Netherlands), Hospital Papa Giovanni XXIII Bergamo and TIGEM, University Federico II, Naples, Italy; Hannover Medical School, Germany; Hopital Antoine-Béclère Paris

Inclusion and exclusion criteria:

- Inclusion criteria: definite or probable Crigler-Najjar syndrome
- Exclusion criteria: None

Study period/begin/stage:

Study start: 01-11-2020

Open for inclusions: yes

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