Initial treatment of idiopathic nephrotic syndrome in children with mycophenolate mofetil vs. prednisone:

A randomized, controlled, multicenter trial (GPN-INTENT Study) (geplanter Studienbeginn 01.02.2014)

**Objective**
To demonstrate that mycophenolate mofetil as initial treatment is not inferior to standard treatment with prednisone related to relapse rate within 24 months

**Key inclusion criteria:**
First episode of SSNS in remission induced by prednisone 60 mg/m² BSA per day; age at beginning of study: ≥ 2 years and ≤ 10 years, which corresponds to the typical age range of patients with SSNS.

**Key exclusion criteria:**
Secondary nephrotic syndrome, estimated glomerular filtration rate (eGFR) <90 ml/min x 1.73 m² BSA, ongoing treatment with glucocorticoids or other immunosuppressive drugs at time of first episode of nephrotic syndrome.

**Primary efficacy endpoint:**
Occurrence of relapse within 24 months after end of initial treatment (non-inferiority)

**Key secondary endpoint(s):**
- Course of the disease: Time from remission to first relapse; number of relapses; mean relapse rate per patient and year; incidence of frequent relapsers.
- Prednisone-associated toxicity: Cumulative prednisone dose (mg/m² BSA); body mass index (standard deviation score); striae; hirsutism; acne; arterial hypertension; disturbances of carbohydrate and lipid metabolism; growth failure; cataract; glaucoma; psychological disturbances
- MMF-associated toxicity: diarrhea; blood cell count disturbances, infections
First episode of steroid-sensitive nephrotic syndrome (SSNS)
- age 2-10 years
- remission induced by prednisone 60 mg/m²/d

remission

period of 5 days

randomization

Control intervention
Prednisone 60 mg/m²/d continued for total of 8 weeks

+ Prednisone 40 mg/m²

Experimental intervention
Mycophenolate mofetil 1200 mg/m²/d
(until 12 weeks total treatment duration)

Prednisone 40 mg/m² alternate day for 2 weeks

End of intervention after a total of 12 weeks

24 months follow-up

End of trial (27 months in total)
Screening: Inclusion criteria fulfilled?
Randomization: within 5 days of fulfilled inclusion criteria (SSNS)
Visit 1: at time of randomization (before start with protocol)
Visit 2: 4 weeks (29-35 d) after start with protocol
Visit 3: 11 weeks (77-84 d) after begin of treatment with prednisone
Visit 4: 3 months after start with protocol
Visit 5: 6 months after start with protocol
Visit 6: 12 months after start with protocol
Visit 7: 18 months after start with protocol
Visit 8: 24 months after start with protocol

<table>
<thead>
<tr>
<th>Screening</th>
<th>Randomization</th>
<th>Visit 1</th>
<th>Visit 2</th>
<th>Visit 3</th>
<th>Visit 4</th>
<th>Visit 5</th>
<th>Visit 6</th>
<th>Visit 7</th>
<th>Visit 8</th>
</tr>
</thead>
<tbody>
<tr>
<td>Med. history</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td>BMI</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td>BP</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td>Urinetest</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td>Blood test</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
<td>X</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>TDM MPA</td>
<td></td>
<td>X</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>24 h ABPM</td>
<td>X</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>X</td>
</tr>
<tr>
<td>HRQoL</td>
<td>X</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>X</td>
</tr>
<tr>
<td>ECG/Echo</td>
<td>X</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>X</td>
</tr>
<tr>
<td>Ophthalmol.</td>
<td>X</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>X</td>
</tr>
</tbody>
</table>
Contact

Dr. M. Benz, München
marcus.benz@med.uni-muenchen.de

Dr. R. Ehren, Köln
rasmus.ehren@uk-koeln.de

Prof. Dr. B. Tönshoff, Heidelberg
burkhard.toenshoff@med.uni-heidelberg.de

PD Dr. L.T. Weber, Köln
lutz.weber@uk-koeln.de

Dr. S. Luntz, KKS Heidelberg
steffen.luntz@med.uni-heidelberg.de

Anja Sander, Biometrikerin, IMBI Heidelberg
sander@imbi.uni-heidelberg.de

Internetseite: www.gpn-intent-studie.de

Auf der Seite finden Sie Infomaterial und Einverständniserklärungen. Diese können als PDF-Datei heruntergeladen werden.