#351

The (great) GETSBI study: unique multi-design RCT study for efficacy of IALURIL® in Interstitial Cystitis with Hunner lesions.

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Aim of the study

Reimbursement of GAG-therapy for bladder pain syndrome / interstitial cystitis with Hunner lesion subtype (IC/BPS HL+) is under debate, evidence regarding efficacy and cost-effectiveness is lacking.

Objective: to determine short and long-term efficacy of Ialuril®* (intra-vesical GAG-therapy) in IC/BPS HL+ with a research design in accordance with a Level 1 evidence (Oxford CEBM)[1].

Study design

The GETSBI study is a multicenter (9 centers) randomized placebo controlled double blind trial, that continues as an aggregated N-of-1 trial** (30 wks) and ends as an open-label intervention study (24 wks). The duration is 54 weeks

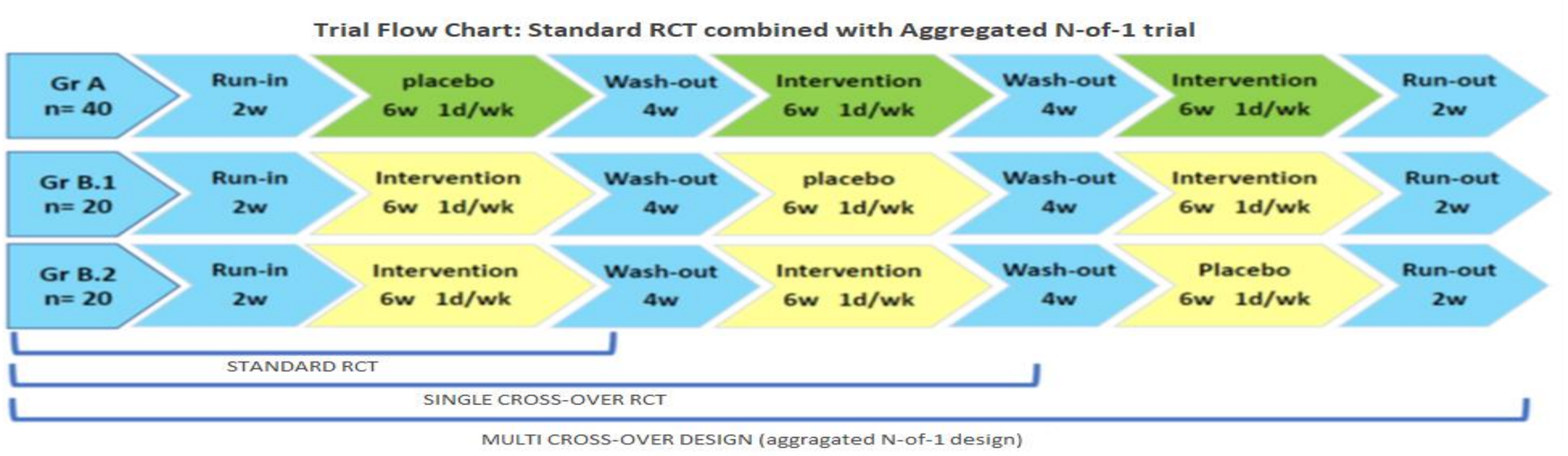
Study population

Symptomatic adult IC/BPS patients (VAS pain score ≥4) with Hunner lesions seen on cystoscopy (≤ 3 months). N=80.

Study outcome

Primary study outcome: improvement on bladder pain (VAS score, scale 0-10)

Main secondary study outcomes: cystoscopic evaluation, cost-effectiveness, Quality of Life and change in self-reported symptoms.



Consideration study design

Randomized Controlled Trial

- ✚ Gold standard in clinical research (Level 1 evidence[1])
- ✚ The use of randomization, double blinding and placebo
- ✚ Suitable for group comparison
- ▬ High number of inclusion needed for power
- ▬ Lack of within-comparison (difficult in heterogenous disease such as IC/BPS).

Aggregated N-of-1 trial

- ✚ Level 1 evidence study design[1]
- ✚ Each patient receives the same amount placebo/intervention
- ✚ The use of randomization, double blinding and placebo
- ✚ Suitable for within-comparison
- ✚ Lower number of inclusion needed for power
- ▬ Less established research design
- ▬ Only possible in chronic disease and non-curing therapies
- ▬ Potential carry-over effects

Implementation in study protocol

In this study design both models are implemented, this is the first use of an aggregated N-of-1 trial methodology in urology. And this study gives a unique insight in how these models compare to each other.

The GETSBI study is a multi-design multicenter randomized placebo-controlled trial to investigate the efficacy of GAG-therapy (Ialuril®) in IC/BPS HL+. The study design is relevant for designing a more adequate study protocol that can obtain a high-level of evidence with fewer patients.

Radboudumc

Currently, 19/80 patients are included in 10 months. Since summer, almost all study centers are open for inclusion.

* Prefill bladder instillation with 1.6% hyaluronic acid & 2% chondroitin sulfate
** A RCT within an individual patient and implements a multi-crossover design.

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References

1. OCEBM Levels of Evidence Working Group*. "The Oxford 2011 Levels of Evidence". Oxford Centre for Evidence-Based Medicine. http://www.cebm.net/index.aspx?o=5653