

Use of the Patient Benefit Index to monitor treatment of chronic spontaneous urticaria with omalizumab

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Little is known about the Patient Benefit Index (PBI) as a tool to monitor omalizumab treatment of chronic spontaneous urticaria (CSU) [1-2]. The aim of this exploratory study was to examine the agreement between PBI and other patient reported outcomes (PROs) used in CSU and to examine what treatment outcomes CSU patients consider most important.

At a tertiary centre, Department of Dermatology, Bispebjerg Hospital, Copenhagen, Denmark 15 consecutive patients with CSU completed PBI, consisting of the Patient Needs Questionnaire (PNQ) before starting treatment with omalizumab 300 mg every fourth week and the Patient Benefit Questionnaire (PBQ) at three months follow-up. The questionnaires consist of 25 questions rated on a five-point scale, from “not at all” to “a lot”. For each question the patient can choose “does not apply to me”. The PNQ concerns the patient’s pre-treatment expectations, whereas the PBQ concerns the patient’s perceived benefit from treatment at follow-up. Based on the PNQ and PBQ a PBI Global Score and five subscale scores can be computed, each having the possible range 0-4. A $PBI \geq 1$ indicates minimal patient-relevant benefit of treatment. Patients also filled Urticaria Activity Score in the past 7 days (UAS7), Dermatology Life Quality Index (DLQI) and a visual analogue scale (VAS) from 0-10 points to document overall disease-related distress with 0 corresponding to “unaffected” and 10 corresponding to the “worst imaginable distress” caused by urticaria.

13 of the patients were women (86.7%) and mean age was 40.5 years. Three months after initiation of omalizumab, UAS7 had decreased 17.4 points (95% CI, 7.7-27.1), $p=0.002$ (from 29.3 to 11.9 points), DLQI had decreased 3.2 points (-5.7-12.1), $p=0.446$ (from 13.5 to 10.3 points), whereas VAS had decreased 4.7 points (2.3-7.2), $p=0.001$ (from 8.4 to 3.6 points). Among seven patients who had complete follow-up data available for PBI, the mean PBI Global Score after

treatment was 3.7 points (SD=0.3) and the fraction of patients with a PBI Global Score ≥ 1 point was 100 %.

After three months we observed high, but statistically insignificant, correlations between the PBI Global Score and the change in the PROs from baseline, particularly VAS ($r=-0.73$, $p=0.103$) and UAS7 ($r=-0.64$, $p=0.173$), and to a lesser degree DLQI ($r=-0.21$, $p=0.741$), whereas change in VAS and UAS7 correlated statistically significantly ($r=0.73$, $p=0.007$). Furthermore, statistically significant correlations were observed between subscale 4 of PBI and change in VAS, UAS7 and the PBI Global Score (Table 1).

Before treatment the most important outcomes for the patients were to: “be free of itching”, mean=3.9, “no longer have skin burning sensations”, mean=3.8, “regain control of the disease”, mean=3.8, “be healed of all skin defects”, mean=3.7 and “get better skin quickly”, mean=3.7, whereas after three months of omalizumab the outcomes that the patients said had improved most were to “be able to lead a normal everyday life”, mean=4.0, “get better skin quickly”, mean=4.0, “no longer have skin burning sensations”, mean=3.7, “regain control of the disease”, mean=3.7 and “have confidence in therapy”, mean=3.7.

The improvements in PROs in this exploratory study are in line with previous findings at our centre [3]. Moreover, all patients had a PBI Global Score ≥ 1 after three months of treatment, indicating that omalizumab has a positive effect on CSU patients, both in terms of reducing disease activity, improving quality of life and to give the patient a subjective feeling of benefit from the treatment. Also, the change in the PROs during treatment, particularly UAS7 and VAS, correlated highly, but not completely, with the PBI Global Score suggesting that PBI potentially adds

important value when evaluating response to treatment in CSU. However, larger studies are needed to more accurately assess CSU patients' subjective needs.

References

1. Zschocke I, Hammelmann U, Augustin M. Therapeutischer Nutzen in der dermatologischen Behandlung. *Hautarzt* 2005;56:839-46.
2. Augustin M, Radtke MA, Zschocke I, Blome C, Behechtnejad J, Schäfer I, Reusch M, Mielke V, Rustenbach SJ. The patient benefit index: a novel approach in patient-defined outcomes measurement for skin diseases. *Arch Dermatol Res* 2009;301:561-1.
3. Ghazanfar MN, Holm JG, Thomsen SF. Effectiveness of omalizumab in chronic spontaneous urticaria assessed with patient-reported outcomes: a prospective study. *J Eur Acad Dermatol Venereol* 2018;32:1761-7.

Table 1. Spearman's correlations between patient-reported outcomes.

	Δ VAS	Δ UAS7	Δ DLQI	PBI Global Score
Δ VAS	1.00			
Δ UAS7	0.73**	1.00		
Δ DLQI	0.34	-0.04	1.00	
PBI Global Score	-0.73	-0.64	-0.21	1.00
SUB1	-0.78	-0.45	-0.26	0.87*
SUB2	0.27	-0.03	0.67	0.41
SUB3	-0.13	0.13	0.00	0.62
SUB4	0.88**	0.88**	-0.32	0.91**
SUB5	-0.13	0.13	0.00	0.41

**Correlation is significant at the 0.01 level, *Correlation is significant at the 0.05 level.

VAS: Visual Analog Scale. UAS7: Urticaria Activity Score in the past 7 days.

DLQI: Dermatology Life Quality Index. PBI: Patient Benefit Index.

SUB1: Subscale 1. Reducing social impairments (Item 11, 13, 14, 15, 16, 17).

SUB2: Subscale 2. Reducing psychological impairments (Item 6, 7, 9, 10, 12).

SUB3: Subscale 3. Reducing impairments due to therapy (Item 18, 19, 20, 21).

SUB4: Subscale 4. Reducing physical impairments (Item 1, 2, 3, 4, 5).

SUB5: Subscale 5. Having confidence in healing (Item 8, 22, 23).