

Patient Benefit Index (PBI)

Questionnaire on Patient-Defined Treatment Objectives and Benefits

User Manual

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1. Description and use

- The questionnaire “Patient Benefit Index” (PBI) measures patient-defined treatment objectives and benefits, particularly in the course of a treatment.
- The PBI can be used in clinical and observational studies and in daily practice.
- The PBI consists of 2 questionnaires. Before therapy, the patient fills in the standardised “**Patient Needs Questionnaire**” on the individual importance of treatment objectives. This reflects his personal preferences with respect to therapeutic benefit. During or after therapy, the patients rates the extent to which the treatment objectives have been achieved in the “**Patient Benefit Questionnaire**”.
- A **global score**, the PBI in the narrower sense, is calculated for each patient by weighing the achievement values of the treatment objectives by their importance to the individual patient.
- Optionally, the PBI can be supplemented by 6 rating scales assessing goal assessment in the following areas: physical well-being, emotional well-being, performance capacity on the job and in everyday living, social contacts, leisure activities, and quality of life.

2. Development

- Background for the development of the PBI was the need for instruments measuring patient-relevant benefit. Preliminary works on benefit measurement have shown that patient benefit in dermatology can only be defined by the patient him-/herself, because the physician’s perspective only partly corresponds with the patient’s perspective. Accordingly, the “patient-relevant benefit” as required by German legislation (IQWiG, Federal Joint Committee, social law) has to be obtained from the individual patient. For this, the PBI questionnaire has been developed specifically for the purpose of evaluating patient-defined benefits in dermatological treatment.

3. Standard and condition-specific versions

The PBI standard version (PBI-S) for use in **all skin diseases** was developed at the CVderm, university clinics of Hamburg. An open survey of n = 100 patients with acute or chronic skin diseases and n = 23 dermatologists yielded an item pool of 213 potentially relevant items. These were reduced to 23 items including only benefits repeatedly mentioned by the patients. Validity of the PBI-S has been confirmed in validation studies on more than 6,000 patients. Two more items were added in the course of further development of the PBI-S.

In addition, a range of **disease-specific** PBI versions have been developed using a standardised procedure:

(1) A sample of usually about 50 patients with the respective disease is surveyed on their disease burdens and treatment objectives.

(2) An expert panel of dermatologists, psychologists, methodologists, and patients discusses the survey results and compares them to the items included in the PBI-S. Then, the disease-specific version is developed.

(3) The resulting questionnaire is validated in a sample of at least n = 100 patients with the respective disease.

All PBI versions are listed in the table in the end of this manual.

4. Languages

Translations of PBI versions have been performed as follows:

1. independent translations by 2 native speakers
2. independent back-translations by 2 German native speakers
3. tabulation of all translations (sentence by sentence) with listing of all differences between translations and differences between back translations and original
4. translators' and methodologists'/authors' conference to find a consensus on the final translation
5. proof reading the final questionnaire by native speaker.

The available languages of the different PBI versions are listed in the table in the end of this manual.

5. Instructions

The PBI is filled in by the patient himself (except for the children's version PBI-AD-K.). The questionnaire is self-explanatory; yet, patients can be supported if they are not able to fill it in by themselves. In this case, the support needs to be documented.

6. Internet-based assessment

The PBI can be assessed via internet, i.e. patients can fill it in on a website. The following has to be considered:

- The instructions are the same as in the paper version, except for the phrase "Please check whether you have marked each statement exactly once" which is omitted in both PNQ and PBQ. The website needs to be programmed so that the patient can send the data (or get to the next part of the survey) only after responding to each item.
- All PNQ items are given on one page and are thus visible to the patient on the same page. Equally, all PBQ items are given on one page.
- The website layout should equal the paper layout as far as possible. In particular, the option "does not apply to me" needs to be visually separated from the other options.

7. Data entry

- The data are coded with numbers (0 to 4; "does/did not apply to me" = 5; missing value = -9) and are entered into a spreadsheet (e.g. Excel) or statistics program (e.g. SPSS). The spreadsheet matrix should be structured as follows: Each row corresponds with one patient and each column corresponds with one variable (=item).
- If an item is answered with two or more "x" or by ticking between two checkboxes, it will be treated as missing.

8. Data analysis

- Part 1: Patient Needs Questionnaire (PNQ). The PNQ consists of the treatment goal items. It is analyzed descriptively. The analysis can include:
 - a) means and standard deviations of all items („does not apply to me“ is coded as 0 for this single-item analysis of the PNQ)
 - b) for each item the percentage of the response „does not apply to me“
 - c) for each item the percentage of responses with high agreement (quite or very)
 - d) percentage of missing values
- Part 2: Patient Benefit Questionnaire (PBQ). The PBQ consists of the benefit items measuring the extent to which the treatment goals have been achieved. It is analyzed descriptively. The analysis can include:
 - a) means and standard deviations of all items („did not apply to me“ being treated as missing here!)
 - b) for each item the percentage of the response „did not apply to me“
 - c) for each item the percentage of responses with high agreement (quite or very)
 - d) percentage of missing values
- The global score PBI is computed for each patient according to the following algorithm. Using this algorithm, the importance of each treatment goal is divided by the sum of all importance values of the respective patient and is multiplied with the goal attainment value. The resulting products are added up.

$$PBI = \sum_{i=1}^k \frac{PNQ_i}{\sum_{i=1}^k PNQ_i} PBQ_i$$

Algorithm for the computation of the PBI global benefit value with k preference items (PNQ) and benefit items (PBQ); possible range of item values and global score values: 0-4.

For score calculation, both "does/did not apply" and "question unanswered" will be treated as missing values. The global score will be calculated using **only those items pairs** (i.e., importance item and benefit item) **for which the patient has given a response other than "does/did not apply" in both PNQ and PBQ**. In other words, importance items for which the corresponding benefit item has been rated as "did not apply" (or vice versa) will not be included in the sum of needs (i.e. the denominator within the algorithm).

- The interpretation of data includes the distribution characteristics of PBI (e.g., mean and standard deviation) and the proportion of patients with $PBI \geq 1$. Patients with $PBI \geq 1$ are considered having at least minimum patient-relevant treatment benefit (based on pilot studies).
- Furthermore, **subscales** of the PBI-S (and for some further PBI versions, too) can be calculated representing different dimensions of treatment benefit. Therefore, the algorithm (see above) is applied to the items of the respective scale. The subscales include the following items (Blome C, Augustin M, Behechtnejad J, Rustenbach SJ: Dimensions of Patient Needs in Dermatology: Subscales of the Patient Benefit Index. Arch Dermatol Res 303(1), 11-17, 2011):

1. **reducing social impairments:**
 - 11 To be less of a burden to relatives and friends
 - 13 To be able to have a normal working life
 - 14 To be able to have more contact with other people
 - 15 To dare to show oneself more
 - 16 To be less burdened in partnership
 - 17 To be able to have a normal sex life
2. **reducing psychological impairments:**
 - 6 To feel less depressed
 - 7 To gain in joy of living
 - 9 To be able to lead a normal everyday life
 - 10 To be more capable in daily life
 - 12 To be able to engage in normal leisure activities
3. **reducing impairments due to therapy:**
 - 18 To be less dependent on doctor and clinic visits
 - 19 To have to spend less time with daily care
 - 20 To have fewer out-of-pocket treatment costs
 - 21 To experience fewer side effects
4. **reducing physical impairments:**
 - 1 To be free of pain
 - 2 To be free of itching
 - 3 To no longer have a burning sensation on the skin
 - 4 To be healed of all skin alterations
 - 5 To be able to sleep better
5. **having confidence in healing:**
 - 8 To have no fear that the disease will progress
 - 22 To find a clear diagnosis and therapy
 - 23 To have confidence in the therapy

- PBI global score and subscales may only be computed if the patient has provided valid data on importance (PNQ) and benefit (PBQ) for at least 75% of the respective treatment goals. In this context, the responses “not at all” and “does/did not apply” count as *valid* data. Thus, a treatment goal is regarded missing if the patient has not responded to the item in the PNQ and/or in the PBI.

9. Psychometric properties of the PBI

The PBI-S is the most widely used PBI version.

The following data are from the PBI-S validation 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 301(8):561-71, 2009).

- Cross-sectional validation in n = 500 patients with 10 distinct skin diseases (50 patients of each of the following diseases: acne vulgaris, atopic eczema, autoimmune diseases of the skin, hand and foot eczema, hair diseases, herpes zoster, hyperhidrosis, psoriasis, ulcus cruris, urticaria). PNQ and PBQ were filled in consecutively with the instruction to rate achieved benefits of the most recently completed therapy.
 - The PNQ was highly reliable/consistent (Cronbach’s alpha = 0.91; part-whole corrected item-total correlations 0.32 to 0.68).
 - High convergent validity was found with respect to treatment success in 6 areas as rated by the patient: physical well-being: r = 0.76; emotional well-being: r = 0.69; performance capacity on the job and in everyday living: r = 0.54; social contacts: r = 0.53; leisure activities: r = 0.53; quality of life: r = 0.71 (all p < 0.001).
 - The PBI discriminates well between different diagnoses: Disease-specific, clinically recognizable patterns of patient-defined needs were found (MANOVA: p < 0.001).

- Longitudinal validation in a treatment study in clinical practice on n = 906 patients with acne vulgaris. The PNQ was recorded at T1 (therapy onset), the PBQ at both T2 (ca. 4 weeks after therapy onset) and at T3 (after 8-12 weeks) under practice conditions
 - The PBI is sensitive to change: The percentage of treatment objectives that had been achieved quite or very well increased from 49.5% at T2 to 62.3% at T3.
 - Under practice conditions, there were between 0.2 and 1.7% missing values, depending on the item.
 - Median convergent validity ($r=0.35$, $p<0.001$) was found with change in the acne-specific quality of life questionnaire “Acne Disability Index“ (ADI).

Information on validity of the single PBI versions can be found in the respective publications, see table below.

10. Contact and license information

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11. Table: Available PBI versions

Condition/diagnosis	PBI version	# of items	Validation paper	Available languages
Standard versions for skin diseases:				

Condition/diagnosis	PBI version	# of items	Validation paper	Available languages
<ul style="list-style-type: none"> • Acne vulgaris • Atopic eczema • Autoimmune skin diseases • Hair diseases • Hand and foot eczema • Herpes zoster • Hyperhidrosis • Psoriasis vulgaris • Ulcus cruris • Urticaria 	PBI-S	25	<p>Validation (different skin diseases): 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 301(8):561-71, 2009</p> <p>Validation (psoriasis): Feuerhahn J, Blome C, Radtke MA, Augustin M. Validation of the Patient Benefit Index for the Assessment of Patient-Relevant Benefit in the Treatment of Psoriasis. Arch Dermatol Res 304(6):433-41, 2012</p> <p>Validation (acne vulgaris): Augustin M, Reich C, Schaefer I, Zschocke I, Rustenbach SJ: Entwicklung und Validierung eines neuen Instrumentes zur Erfassung patientendefinierten Nutzens in der Therapie der Akne. J Dtsch Dermatol Ges 6(2): 113–20, 2008. --- Article is also available in English (Development and validation of a new instrument for the assessment of patient-defined benefit in the treatment of acne).</p> <p>Subscales: Blome C, Augustin M, Behechtnejad J, Rustenbach SJ: Dimensions of Patient Needs in Dermatology: Subscales of the Patient Benefit Index. Arch Dermatol Res 303(1), 11-17, 2011</p>	Chinese (Taiwan), Croatian (Croatia), Czech (Czech Republic), Danish (Denmark), Dutch (Netherlands), Dutch (Belgium), English (Australia), English (Canada), English (UK), English (USA), French (Canada), French (France & Belgium), French (Switzerland), German (Austria), German (Germany), German (Switzerland), Greek (Greece), Hungarian (Hungary), Italian (Italy), Italian (Switzerland), Japanese (Japan), Korean (Korea), Polish (Poland), Russian (Russia), Slovenian (Slovenia), Spanish (Spain), Spanish (USA), Swedish (Sweden), Ukrainian (Ukraine)

Condition/diagnosis	PBI version	# of items	Validation paper	Available languages
<ul style="list-style-type: none"> • Atopic dermatitis • Psoriasis • Ulcus cruris • Vitiligo 	PBI 2.0	12	Topp J, Augustin M, von Usslar K, Gosau R, Reich K, Reusch M, Blome C (2018). Measuring Patient Needs and Benefits in Dermatology using the 'Patient Benefit Index 2.0' – A Validation Study. <i>Acta Derm Venereol.</i> 99(2):211-7, 2019	German (Germany)
Condition-specific versions:				
Acne inversa / Hidradenitis suppurativa	PBI-HS	26	<i>Publication under submission</i>	German (Germany)
Actinic keratosis	PBI-AK			German (Germany)
Aged skin	PBI-AH	20	Lohrberg D, Blaak J, Liebsch J, Staib P, Wohlfart R, Lüttje D, Schürer NY, Augustin M, Blome C. Development and Validation of the Patient Benefit Index for the Dermatocosmetic Treatment of Aged Skin <i>Arch Dermatol Res</i> 308(5):319-24, 2016	German (Germany)
Allergic rhinitis	PBI-AR	25	<p>Franzke N, Schäfer I, Jost K, Blome C, Rustenbach SJ, Reich K, Reusch M, Maurer M, Augustin M: A new instrument for the assessment of patient-defined benefit in the treatment of allergic rhinitis. <i>Allergy.</i> May;66(5):665-70, 2011</p> <p>Demoly P, Aubier M, de Blay F, Wessel F, Clerson P, Maigret P: Evaluation of patients' expectations and benefits in the treatment of allergic rhinitis with a new tool: the patient benefit index - the benefica study. <i>Allergy Asthma Clin Immunol.</i> Feb 26;11(1):8, 2015</p>	German (Germany), Russian (Russia), Dutch (Netherlands), Italian (Italy), French (France), Spanish (Spain), English (UK), Polish (Poland)
Allergic rhinitis in children	PBI-AR-K	19	<i>Validation paper under preparation</i>	German (Germany)
Allergic rhinoconjunctivitis: special version for evaluation of specific immunotherapy	PBI-SIT	33		German (Germany)

Condition/diagnosis	PBI version	# of items	Validation paper	Available languages
Atopic dermatitis in children (to be filled in by child or parents, depending on the child's age)	PBI-AD-K	23		German (Germany), Russian (Russia), Dutch (Netherlands), Italian (Italy), French (France), Spanish (Spain), English (UK), Polish (Poland)
Chronic hand eczema	PBI-HE	23	Blome C, Maares J, Diepgen T, Rustenbach SJ, Augustin M: Measurement of patient-relevant benefits in the treatment of chronic hand eczema – a novel approach. Contact Dermatitis 61: 39-45, 2009	German (Germany), Russian (Russia), Dutch (Netherlands), Italian (Italy), French (France), Spanish (Spain), English (UK), Polish (Poland)
Chronic wounds	PBI-W	22	Augustin M, Blome C, Zschocke I, Schäfer I, Koenig S, Rustenbach SJ, Herberger K: Benefit evaluation in the therapy of chronic wounds from the patients' perspective-development and validation of a new method. Wound Repair Regen: 20(1):8-14, 2012	German (Germany), Russian (Russia), Dutch (Netherlands), Italian (Italy), French (France), Spanish (Spain), English (UK), Polish (Poland)
Chronic wounds, special version for evaluation of ultrasound treatment	PBI-UAW	24	<i>Instrument was used in:</i> Herberger K, Franzke N, Blome C, Kirsten N, Augustin M: Efficacy, Tolerability and Patient Benefit of Ultrasound-Assisted Wound Treatment versus Surgical Debridement: A Randomized Clinical Study. Dermatology: 222(3):244-9, 2011	German (Germany), Italian (Italy), French (France), Spanish (Spain), English (UK), Polish (Poland)
Cosmetic indications	PBI-K	24	Augustin M, Schäfer I, Rabini S, Lee-Seifert C, Radtke M, Rustenbach SJ: Assessing the value of supportive skin care: development and validation of an instrument for evaluating patient-relevant benefit. Dermatology 218(3):255-9, 2009	German (Germany), Russian (Russia), Dutch (Netherlands), Italian (Italy), French (France), Spanish (Spain), English (UK), Polish (Poland)

Condition/diagnosis	PBI version	# of items	Validation paper	Available languages
Interstitial cystitis	PBI-IZ	22	<i>Manuscript in preparation</i>	German (Germany), Polish (Poland)
Lymphedema	PBI-L	23	Blome C, Augustin M, Heyer K, Knöfel J, Cornelsen H, Purwins S, Herberger K: Evaluation of patient-relevant outcomes of lymphedema and lipedema treatment: development and validation of a new benefit tool. <i>Eur J Vasc Endovasc Surg</i> 47(1):100-7, 2013 Duygu E, Bakar Y, Keser I. An Important Tool in Lymphedema Management: Validation of Turkish Version of the Patient Benefit Index-Lymphedema. <i>Lymphat Res Biol</i> 18(1):49-5, 2020	German (Germany), English (UK), Turkish (Turkey), Polish (Poland)
Multiple sclerosis	PBI-MS	27	Beckmann H, Augustin M, Heesen C, Poettgen J, Blome C: Benefit evaluation in multiple sclerosis relapse treatment from the patients' perspective - Development and validation of a new questionnaire. <i>Mult Scler Relat Disord.</i> 28:256-61, 2019	German (Germany)
Nail psoriasis	NAPPA-PBI	24	Augustin M, Blome C, Costanzo A, Dauden E, Ferrandiz C, Girolomoni G, Gniadecki R, Iversen L, Menter A, Michaelis-Wittern K, Morita A, Nakagawa H, Reich K: Nail Assessment in Psoriasis and Psoriatic Arthritis (NAPPA): Development and Validation of a Tool for Assessment of Nail Psoriasis Outcomes. <i>Br J Dermatol</i> 170:591-8, 2014	German (Germany), Italian (Italy), Spanish (Spain), English (UK), Portuguese (Portugal), Greek (Greece), Danish (Denmark), Japanese (Japan), Polish (Poland)
Neurofibromatosis	PBI-NF	28	<i>Manuscript in preparation</i>	German (Germany), English (UK), Polish (Poland)
Peripheral artery occlusive disease	PBI-POD	12	Zander N, Demirel EB, Augustin M, Sommer R, Debus ES, Breuer P, Blome C (2018). Development and validation of the Patient Benefit Index for peripheral arterial disease. <i>VASA</i> 47(3):219-226, 2018	German (Germany), English (UK)

Condition/diagnosis	PBI version	# of items	Validation paper	Available languages
Pruritus	PBI-P	27	Blome C, Augustin M, Siepmann D, Phan NQ, Rustenbach SJ, Ständer S. Measuring patient-relevant benefits in pruritus treatment: development and validation of a specific outcomes tool. <i>Br J Dermatol</i> 161(5):1143-8, 2009	German (Germany, Austria), Russian (Russia), Dutch (Netherlands), Italian (Italy), French (France), Spanish (Spain), Spanish (USA), English (UK), English (USA), Polish (Poland)
Rheumatoid arthritis	PBI-Rheuma	20	<i>Instrument currently under validation</i>	German (Germany)
Rosacea	PBI-RO	23	<i>Manuscript in preparation</i>	German (Germany), Russian (Russia), Dutch (Netherlands), Italian (Italy), French (France), Spanish (Spain), English (UK), Polish (Poland)
Venous diseases	PBI-V	23	<i>Manuscript in preparation</i>	German (Germany), Russian (Russia), Dutch (Netherlands), Italian (Italy), French (France), Spanish (Spain), English (UK), Polish (Poland)
Vitiligo	PBI-Vit	26	Augustin M, Gajur AI, Reich C, Rustenbach SJ, Schaefer I. Benefit Evaluation in Vitiligo treatment: Development and Validation of a Patient-Defined Outcomes Questionnaire. <i>Dermatology</i> 217: 101-106, 2008	German (Germany), Russian (Russia), Dutch (Netherlands), Italian (Italy), French (France), Spanish (Spain), English (UK), Polish (Poland)