Sarilumab
(polyarticular juvenile idiopathic arthritis)

Benefit assessment according to §35a SGB V[1]

EXTRACT

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Medical and scientific advice

No advisor on medical and scientific questions was involved in the present dossier assessment.

Patient and family involvement

No patients or families were involved in the present dossier assessment.

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Part I: Benefit assessment

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[2] Table numbers start with “2” as numbering follows that of the full dossier assessment.

I List of abbreviations

Abbreviation	Meaning
ACT	appropriate comparator therapy
G-BA	Gemeinsamer Bundesausschuss (Federal Joint Committee)
IQWiG	Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen (Institute for Quality and Efficiency in Health Care)
MTX	methotrexate
RCT	randomized controlled trial
SGB	Sozialgesetzbuch (Social Code Book)

I 1 Executive summary of the benefit assessment

Background

In accordance with § 35a Social Code Book V, the Federal Joint Committee (G-BA) has commissioned the Institute for Quality and Efficiency in Health Care (IQWiG) to assess the benefit of the drug sarilumab. The assessment is based on a dossier compiled by the pharmaceutical company (hereinafter referred to as the "company"). The dossier was sent to IQWiG on 04 February 2025.

Research question

The research questions presented in Table 2 were defined in accordance with the appropriate comparator therapy (ACT) specified by the G-BA.

Table 2: Research questions for the benefit assessment of sarilumab

Research question	Therapeutic indication	ACT[a]
1	Children and adolescents aged 2 years and older with active polyarticular juvenile idiopathic arthritis (rheumatoid factor positive [RF+] or negative [RF-] polyarthritis and extended oligoarthritis) who have responded inadequately to previous treatment with cDMARDs[b]	A bDMARD (adalimumab or etanercept or golimumab or tocilizumab) in combination with MTX; if applicable as monotherapy under consideration of the respective approval status in case of MTX intolerance or unsuitability
2	Children and adolescents aged 2 years and older with active polyarticular juvenile idiopathic arthritis (RF+ or RF- polyarthritis and extended oligoarthritis) who have responded inadequately to previous therapy with bDMARDs[b]	A bDMARD (abatacept or adalimumab or etanercept or golimumab or tocilizumab) in combination with MTX; if applicable as monotherapy under consideration of the respective approval status in case of MTX intolerance or unsuitability depending on prior therapy[c]

a. Presented are the respective ACTs specified by the G-BA.
b. According to the G-BA, it is assumed that the patients covered by the therapeutic indication are not (no longer) eligible for (symptomatic) treatment with nonsteroidal anti-inflammatory drugs (NSAIDs) and/or corticosteroids. The use of glucocorticoids (systemic and/or intra-articular) should be possible as part of a relapse therapy.
c. It is assumed that when selecting the comparator, a switch is made to a biologic disease-modifying antirheumatic drug (bDMARD) that has not yet been used as part of the previous therapy. Unchanged continuation of an inadequate (pre)treatment does not correspond to the ACT.

bDMARD: biologic DMARD; csDMARD: conventional synthetic DMARD; DMARD: disease-modifying antirheumatic drug; G-BA: Federal Joint Committee; MTX: methotrexate; NSAID: nonsteroidal anti-inflammatory drug

The company followed the ACT specified by the G-BA.

The assessment was conducted by means of patient-relevant outcomes on the basis of the data provided by the company in the dossier. Randomized controlled trials (RCTs) with a minimum duration of 24 weeks are used for deriving any added benefit.

Results

The check for completeness of the study pool identified no relevant RCTs on the direct comparison of sarilumab with the G-BA's ACT.

Results on added benefit

Since no relevant study is available for the benefit assessment, there is no hint of an added benefit of sarilumab in comparison with the ACT; an added benefit is therefore not proven.

Probability and extent of added benefit, patient groups with therapeutically important added benefit[3]

Table 3 presents a summary of the probability and extent of the added benefit of sarilumab.

[3] On the basis of the scientific data analysed, IQWiG draws conclusions on the (added) benefit or harm of an intervention for each patient-relevant outcome. Depending on the number of studies analysed, the certainty of their results, and the direction and statistical significance of treatment effects, conclusions on the probability of (added) benefit or harm are graded into 4 categories: (1) "proof", (2) "indication", (3) "hint", or (4) none of the first 3 categories applies (i.e., no data available or conclusions 1 to 3 cannot be drawn from the available data). The extent of added benefit or harm is graded into 3 categories: (1) major, (2) considerable, (3) minor (in addition, 3 further categories may apply: non-quantifiable extent of added benefit, added benefit not proven, or less benefit). For further details see [1,2].

Table 3: Sarilumab – probability and extent of added benefit

Research question	**Therapeutic indication**	**ACT[a]**	**Probability and extent of added benefit**
1	Children and adolescents aged 2 years and older with active polyarticular juvenile idiopathic arthritis (RF+ or RF- polyarthritis and extended oligoarthritis) who have responded inadequately to previous therapy with csDMARDs[b]	A bDMARD (adalimumab or etanercept or golimumab or tocilizumab) in combination with MTX; if applicable as monotherapy under consideration of the respective approval status in case of MTX intolerance or unsuitability	Added benefit not proven
2	Children and adolescents aged 2 years and older with active polyarticular juvenile idiopathic arthritis (RF+ or RF- polyarthritis and extended oligoarthritis) who have responded inadequately to previous therapy with bDMARDs[b]	A bDMARD (abatacept or adalimumab or etanercept or golimumab or tocilizumab) in combination with MTX; if applicable as monotherapy under consideration of the respective approval status in case of MTX intolerance or unsuitability depending on prior therapy[c]	Added benefit not proven

a. Presented are the respective ACTs specified by the G-BA.
b. According to the G-BA, it is assumed that the patients covered by the therapeutic indication are not (no longer) eligible for (symptomatic) treatment with nonsteroidal anti-inflammatory drugs (NSAIDs) and/or corticosteroids. The use of glucocorticoids (systemic and/or intra-articular) should be possible as part of a relapse therapy.
c. It is assumed that when selecting the comparator, a switch is made to a bDMARD that has not yet been used as part of the previous therapy. Unchanged continuation of an inadequate (pre)treatment does not correspond to the ACT.

bDMARD: biologic DMARD; csDMARD: conventional synthetic DMARD; DMARD: disease-modifying antirheumatic drug; G-BA: Federal Joint Committee; MTX: methotrexate; NSAID: nonsteroidal anti-inflammatory drug

The G-BA decides on the added benefit.

I 2 Research question

Aim of this report is the assessment of the added benefit of sarilumab in comparison with the ACT in children and adolescents 2 years of age and older with active juvenile idiopathic arthritis (pJIA; RF+ or RF− polyarthritis and extended oligoarthritis) who have responded inadequately to previous therapy with csDMARDs. Sarilumab may be used as monotherapy or in combination with methotrexate (MTX).

The research questions presented in Table 4 were defined in accordance with the ACT specified by the G-BA.

Table 4: Research questions for the benefit assessment of sarilumab

Research question	Therapeutic indication	ACT[a]
1	Children and adolescents aged 2 years and older with active polyarticular juvenile idiopathic arthritis (RF+ or RF- polyarthritis and extended oligoarthritis) who have responded inadequately to previous therapy with csDMARDs[b]	A bDMARD (adalimumab or etanercept or golimumab or tocilizumab) in combination with MTX; if applicable as monotherapy under consideration of the respective approval status in case of MTX intolerance or unsuitability
2	Children and adolescents aged 2 years and older with active polyarticular juvenile idiopathic arthritis (RF+ or RF- polyarthritis and extended oligoarthritis) who have responded inadequately to previous therapy with bDMARDs[b]	A bDMARD (abatacept or adalimumab or etanercept or golimumab or tocilizumab) in combination with MTX; if applicable as monotherapy under consideration of the respective approval status in case of MTX intolerance or unsuitability depending on prior therapy[c]

a. Presented are the respective ACTs specified by the G-BA.
b. According to the G-BA, it is assumed that the patients covered by the therapeutic indication are not (no longer) eligible for (symptomatic) treatment with nonsteroidal anti-inflammatory drugs (NSAIDs) and/or corticosteroids. The use of glucocorticoids (systemic and/or intra-articular) should be possible as part of a relapse therapy.
c. It is assumed that when selecting the comparator, a switch is made to a bDMARD that has not yet been used as part of the previous therapy. Unchanged continuation of an inadequate (pre)treatment does not correspond to the ACT.

bDMARD: biologic DMARD; csDMARD: conventional synthetic DMARD; DMARD: disease-modifying antirheumatic drug; G-BA: Federal Joint Committee; MTX: methotrexate; NSAID: nonsteroidal anti-inflammatory drug

The company followed the ACT specified by the G-BA.

The assessment was conducted by means of patient-relevant outcomes on the basis of the data provided by the company in the dossier. RCTs with a minimum duration of 24 weeks are used for deriving any added benefit. This did not concur with the inclusion criteria used by the company, which included RCTs with a minimum duration of 3 months.

I 3 Information retrieval and study pool

The study pool for the assessment was compiled on the basis of the following information:

Sources used by the company in the dossier:

- Study list on sarilumab (status: 22 November 2024)
- Bibliographical literature search on sarilumab (last search on 22 November 2024)
- Search in trial registries/trial results databases for studies on quizartinib (last search on 22 November 2024)
- Search on the G-BA website for sarilumab (last search on 22 November 2024)

To check the completeness of the study pool:

- Search in trial registries for studies on sarilumab (last search on 27 February 2025); for search strategies, see I Appendix A of the full dossier assessment

No relevant study was identified from the check.

The company supports the description of the medical benefit with the approval study SKYPP [3], a non-randomized, non-controlled, open-label phase IIB study with a dose-finding and an extension phase. The SKYPP study does not include any comparisons with the ACTs; therefore, data on the comparison of sarilumab with the comparator therapies specified by the G-BA are not available.

I 4 Results on added benefit

Since no relevant study is available for the benefit assessment, there is no hint of an added benefit of sarilumab in comparison with the ACT; an added benefit is therefore not proven.

I 5 Probability and extent of added benefit

The result of the assessment of the added benefit of sarilumab in comparison with the ACT is summarized in Table 5.

Table 5: Sarilumab – probability and extent of added benefit

Research question	Therapeutic indication	ACT[a]	Probability and extent of added benefit
1	Children and adolescents aged 2 years and older with active polyarticular juvenile idiopathic arthritis (RF+ or RF- polyarthritis and extended oligoarthritis) who have responded inadequately to previous therapy with csDMARDs[b]	A bDMARD (adalimumab or etanercept or golimumab or tocilizumab) in combination with MTX; if applicable as monotherapy under consideration of the respective approval status in case of MTX intolerance or unsuitability	Added benefit not proven
2	Children and adolescents aged 2 years and older with active polyarticular juvenile idiopathic arthritis (RF+ or RF- polyarthritis and extended oligoarthritis) who have responded inadequately to previous therapy with bDMARDs[b]	A bDMARD (abatacept or adalimumab or etanercept or golimumab or tocilizumab) in combination with MTX; if applicable as monotherapy under consideration of the respective approval status in case of MTX intolerance or unsuitability depending on prior therapy[c]	Added benefit not proven

a. Presented are the respective ACTs specified by the G-BA.
b. According to the G-BA, it is assumed that the patients covered by the therapeutic indication are not (no longer) eligible for (symptomatic) treatment with nonsteroidal anti-inflammatory drugs (NSAIDs) and/or corticosteroids. The use of glucocorticoids (systemic and/or intra-articular) should be possible as part of a relapse therapy.
c. It is assumed that when selecting the comparator, a switch is made to a bDMARD that has not yet been used as part of the previous therapy. Unchanged continuation of an inadequate (pre)treatment does not correspond to the ACT.

bDMARD: biologic DMARD; csDMARD: conventional synthetic DMARD; DMARD: disease-modifying antirheumatic drug; G-BA: Federal Joint Committee; MTX: methotrexate; NSAID: nonsteroidal anti-inflammatory drug

The assessment described above concurs with the company's assessment.

The G-BA decides on the added benefit.

The reference list contains citations provided by the company in which bibliographical information may be missing.

I 6 References for English extract

Please see full dossier assessment for full reference list.

The reference list contains citations provided by the company in which bibliographical information may be missing.

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