Cipaglucosidase alfa (Pompe disease 1)

Addendum to Project A23-79 (dossier assessment)[1]

ADDENDUM

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Abbreviation	Meaning
6MWT	6-minute walk test
ACT	appropriate comparator therapy
GAA	acid α-glucosidase
G-BA	Gemeinsamer Bundesausschuss (Federal Joint Committee)
IQWiG	Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen (Institute for Quality and Efficiency in Health Care)
LOPD	late-onset Pompe disease
NRI	non-response imputation
PROMIS	Patient Reported Outcome Measurement Information System
RCT	randomized controlled trial
R-PAct	Rasch-built Pompe-specific Activity
SGB	Sozialgesetzbuch (Social Code Book)
SGIC	Subject's Global Impression of Change

1 Background

On 12 December 2023, the Federal Joint Committee (G-BA) commissioned the Institute for Quality and Efficiency in Health Care (IQWiG) to conduct supplementary assessments for Project A23-79 (cipaglucosidase alfa – Benefit assessment according to § 35a Social Code Book V) [1].

The commission comprises the assessment of the following analyses presented by the pharmaceutical company (hereinafter referred to as "the company") in the commenting procedure [2], taking into account the information provided in the dossier [3]:

- Subsequently submitted data on the outcomes of the total population of the PROPEL study recorded using the Patient Reported Outcome Measurement Information System (PROMIS) and Rasch-built Pompe-specific Activity (R-Pact) scale

The responsibility for the present assessment and the assessment result lies exclusively with IQWiG. The assessment is forwarded to the G-BA. The G-BA decides on the added benefit.

2 Assessment

The randomized controlled trial (RCT) PROPEL was included for the benefit assessment of cipaglucosidase alfa in combination with miglustat (hereafter referred to as "cipaglucosidase alfa + miglustat") versus the appropriate comparator therapy (ACT) alglucosidase alfa in adult patients with late-onset Pompe disease (acid α-glucosidase [GAA] deficiency [LOPD]). A detailed description of the study can be found in dossier assessment A23-79 [1].

As commissioned, the analyses subsequently submitted by the company in the commenting procedure [2] on the outcomes "physical functioning" (recorded with R-PAct and PROMIS Physical Function), "fatigue" (recorded with PROMIS Fatigue), "dyspnoea" (recorded with PROMIS Dyspnea Severity) and "function of the upper extremities" (recorded with PROMIS Upper Extremity) are assessed below. In the present assessment, a deterioration by the respective response threshold (≥ 15% of the instrument's scale range) at Week 52 is used as a suitable operationalization (see also dossier assessment A23-79).

2.1 Responder analyses on patient-reported outcomes recorded using R-PAct and PROMIS

Time of analysis at Week 52 relevant for the benefit assessment

The dossier [3] provided responder analyses on the assessment time up to Week 52 for the outcomes of physical functioning (R-PAct, PROMIS Physical Functioning), fatigue (PROMIS-Fatigue), dyspnoea (PROMIS Dyspnea Severity) and function of the upper extremities (PROMIS Upper Extremity). In the company's analyses on deterioration, a patient was considered a responder if he or she showed a deterioration by the response criterion at (any) time during the course of the study (up to week 52). In the present therapeutic indication of a chronic, progressive disease, however, it is relevant to consider the outcomes as late as possible (i.e. in the PROPEL study at the end of the study at Week 52). Therefore, the responder analyses presented by the company for the outcomes recorded with R-PAct and PROMIS are unsuitable for the benefit assessment. In its comments, the company presented responder analyses for the outcomes recorded using R-PAct and PROMIS at the time of analysis at Week 52. This is appropriate.

Approach of the company for transforming the raw values of the R-PAct and PROMIS questionnaires

Furthermore, the company based its analyses for the outcomes in the dossier recorded using R-PAct and PROMIS on the raw values and - contrary to the procedure described in the publication on R-PAct [4] and the PROMIS manuals [5-7] - did not transform the values. Therefore, the analyses presented by the company in the dossier for the outcomes recorded with R-PAct and PROMIS are unsuitable for the benefit assessment.

As part of the commenting procedure, the company presented post hoc responder analyses based on transformed values at Week 52 for the outcomes recorded using PROMIS and R-PAct.

According to the PROMIS manuals [5-7], there are - as already described in dossier assessment A23-79 - two methods for transforming the raw values: firstly, manual transformation via the conversion table in the corresponding manual, and secondly, the so-called "response scoring pattern", which enables the generation of a final, transformed value even if values for individual or several items are missing (e.g. via the HealthMeasures Scoring Service [8]). In the commenting procedure, the company stated that it had not performed the transformation using a scoring service (i.e. response scoring pattern). Instead, it used the conversion tables of the corresponding PROMIS manuals [5-7] to transform the raw values. According to the company, analyses were only possible for fully completed questionnaires. However, the procedure chosen by the company for the transformation means that patients with missing values for individual or several items are not included in the analyses with a value generated in the scoring. For these patients, the company then replaced the missing total score by means of non-response imputation (NRI). In some cases, this results in very high percentages of missing or imputed values (see Table 1). In the outcome of dyspnoea (PROMIS Dyspnea Severity), missing values were imputed by means of NRI for 60% of patients. The results for the outcome dyspnoea are not suitable for the benefit assessment due to the too high percentage of imputed values. For the other outcomes assessed using PROMIS, the proportion of imputed values ranges between 7 % and 30 % (see Table 1), and the respective results are used for the benefit assessment in the present data situation. However, the extent of imputed values has been taken into account in the assessment of the risk of bias of results for the individual outcomes (see Section 2.1.1).

According to the publication [4], for the R-PAct, the raw values are transformed manually using a conversion table. A transformation of the raw values using a conversion table is only possible if all questions of the questionnaire have been completed by the patient. In its comments, the company refers to the publication on the R-PAct [4] and states that analyses could only be performed for fully completed questionnaires. Therefore, the company’s approach for transforming the raw values of the R-PAct questionnaire is adequate. However, there is a high percentage of missing values (24%; see Table 1), which the company replaces - as with the PROMIS instruments - using NRIs. The extent of imputed values has been taken into account in the assessment of the risk of bias of results (see Section 2.1.1).

Table 1: Overview of imputed values in the responder analyses for the outcomes of the PROPEL study recorded using R-PAct and PROMIS

Outcome category (analysis date) **outcome** **imputation**	**Cipaglucosidase alfa + miglustat** **N = 85**	**Alglucosidase alfa + placebo** **N = 38**
Morbidity (at Week 52)		
Physical functioning		
R-PAct		
N (%) NRI-imputed values	23 (27.1)	6 (15.8)
PROMIS Physical Function		
N (%) NRI-imputed values	15 (17.6)	7 (18.4)
Fatigue (PROMIS Fatigue)		
N (%) NRI-imputed values	6 (7.1)	2 (5.3)
Dyspnoea (PROMIS Dyspnea Severity)		
N (%) NRI-imputed values	50 (58.8)	24 (63.2)
Function of the upper extremities (PROMIS Upper Extremity)		
N (%) NRI-imputed values	28 (32.9)	9 (23.7)

N: Number of analysed patients; n: Number of imputed values; NRI: non-response imputation; PROMIS: Patient Reported Outcome Measurement Information System; R-PAct: Rasch-built Pompe-specific Activity

Response criterion 15% was relevant for the benefit assessment

In its analyses, the company considered 15% of the respective scale range (based on the transformed values) as the response threshold. Patients are counted as responders by the company if they have exceeded the following response thresholds at Week 52: ≥ 15 points for the R-PAct (scale range 0 to 100), ≥ 8.025 points for the PROMIS Physical Function (scale range 9.2 to 62.7), ≥ 6.705 points for the PROMIS Fatigue (scale range 33.1 to 77.8), ≥ 7.23 points for the PROMIS Dyspnea Severity (scale range 27.7 to 75.9) or ≥ 6.285 points for the PROMIS Upper Extremity (scale range 16.3 to 58.2). Patients were categorized as non-responders by the company if they did not exceed the respective threshold value at Week 52 or did not have a value at Week 52.

The response criterion of 15% of the respective scale range, which was used in the analyses presented by the company, fulfils the requirements for response criteria of reflecting with sufficient certainty a change that is perceivable for patients, as defined by the *General Methods* of the Institute [9]. Therefore, the analyses of this response threshold are each used for the benefit assessment (with the exception of the data on the outcome “dyspnoea” [PROMIS Dyspnea Severity; see previous comments]).

2.1.1 Risk of bias

The result for the outcome “dyspnoea” (PROMIS Dyspnea Severity) is not used due to the too high percentage of imputed values (see Table 1); an assessment of the risk of bias is therefore omitted.

For the result on the outcome “fatigue” (PROMIS Fatigue), the risk of bias is rated as low.

The risk of bias of the results on the outcomes of physical functioning (R-PAct, PROMIS Physical Function) and function of the upper extremities (PROMIS Upper Extremity) is rated as high due to the high proportion of substituted values (see Table 1).

2.1.2 Results

The results on the outcomes of physical functioning (R-PAct, PROMIS Physical Function), fatigue (PROMIS Fatigue) and function of the upper extremities (PROMIS Upper Extremity) are shown in Table 2. As described in the previous sections, no suitable data are available for the outcome of dyspnoea (PROMIS Dyspnea Severity).

Table 2: Results (morbidity, dichotomous) – RCT, direct comparison: cipaglucosidase alfa + miglustat versus alglucosidase alfa + placebo

Study outcome category outcome	Cipaglucosudase alfa + miglustat		Alglucosidase alfa + placebo		Cipaglucosidase alfa + miglustat vs. alglucosidase alfa + placebo
	N	patients with event n (%)	N	patients with event n (%)	RR [95% CI]; p-value[a]
PROPEL					
Morbidity (at Week 52)					
Physical functioning					
R-PAct (worsening[b])	85	1 (1)	38	0 (0)	–[c]
PROMIS Physical Function (worsening[d])	85	0 (0)	38	1 (3)	–[c]
Fatigue (PROMIS Fatigue; worsening[e])	85	5 (6)	38	3 (8)	0.78 [0.18; 3.39]; 0.739
Dyspnoea (PROMIS Dyspnea Severity; worsening[f])	No suitable data[g]				
Function of the upper extremities (PROMIS Upper Extremity; worsening[h])	85	4 (5)	38	0 (0)	1.41 [0.36; 5.54]; 0.618

a. The company provides no information on the analysis method; presumably analogous to the analyses in its dossier "CMH method": stratified by distance travelled in theat baseline and enzyme replacement therapy status; if 1 zero cell occurred in 1 stratum in the corresponding 2x2 table, a correction value of 0.5 was added to each of the cell frequencies of the stratum; missing values were imputed by NRI.
b. A decrease by ≥ 15 points from baseline is regarded as a clinically relevant deterioration (scale range 0 to 100).
c. No presentation of the effect estimate, as no person in one treatment arm and only 1 person in the other treatment arm had an event.
d. A decrease by ≥ 8.025 points from baseline is defined as a clinically relevant deterioration (scale range 9.2 to 62.7).
e. A score increase by ≥ 6.705 points from baseline is defined as a clinically relevant deterioration (scale range 33.1 to 77.8).
f. A score increase by ≥ 7.23 points from baseline is defined as a clinically relevant deterioration (scale range 27.7 to 75.9).
g. The results for the outcome "dyspnoea" are not suitable for the benefit assessment due to the too high percentage of imputed values (Section 2.1 of the present addendum).
A decrease by ≥ 6.285 points from baseline is defined as a clinically relevant deterioration (scale range 16.3 to 58.2).

6MWT: 6-minute walk test; CI: confidence interval; CMH: Cochran-Mantel-Haenszel; n: number of patients with (at least 1) event; N: number of analysed patients; NRI: non-response imputation; PROMIS: Patient Reported Outcome Measurement Information System; RCT: randomized controlled trial; R-PAct: Rasch-built Pompe-specific Activity; RR: relative risk

Based on the available information, at most hints, e.g. of added benefit, can be derived for the outcomes of physical functioning (R-PAct, PROMIS Physical Function) and function of the upper extremities (PROMIS Upper Extremity) due to the high risk of bias. For the outcome of fatigue (PROMIS Fatigue), at most indications, e.g. of an added benefit, can be determined.

Morbidity

Physical functioning (R-PAct, PROMIS Physical Function)

No statistically significant difference between treatment arms was shown for the outcome of physical functioning (R-PAct, PROMIS Physical Function). There is no hint of an added benefit of cipaglucosidase alfa + miglustat in comparison with alglucosidase alfa; an added benefit is therefore not proven.

Fatigue (PROMIS Fatigue)

No statistically significant difference between treatment arms was found for the outcome of fatigue (PROMIS Fatigue). There is no hint of an added benefit of cipaglucosidase alfa + miglustat in comparison with alglucosidase alfa; an added benefit is therefore not proven.

Dyspnoea (PROMIS Dyspnea Severity)

No suitable data are available for the outcome of dyspnoea (PROMIS Dyspnea Severity). There is no hint of an added benefit of cipaglucosidase alfa + miglustat in comparison with alglucosidase alfa; an added benefit is therefore not proven.

Function of the upper extremities (PROMIS Upper Extremity)

No statistically significant difference between treatment arms was shown for the outcome "function of the upper extremities" (PROMIS Upper Extremity). There is no hint of an added benefit of cipaglucosidase alfa + miglustat in comparison with alglucosidase alfa; an added benefit is therefore not proven.

2.1.3 Subgroups and other effect modifiers

For the present benefit assessment, the following subgroup characteristics are relevant (see dossier assessment A23-79 [1]):

- Age (≥ 18 to < 35 years versus ≥ 35 to < 50 years versus ≥ 50 to < 65 years versus ≥ 65 years)
- Sex (female versus male)
- Distance travelled in the (6-minute walk test) 6MWT at baseline (≥ 75 to < 150 m vs. ≥ 150 to < 400 m vs. ≥ 400 m)
- Enzyme replacement therapy status (naive vs. pretreated)

Interaction tests are performed when at least 10 patients per subgroup are included in the analysis. For binary data, there must also be at least 10 events in at least one subgroup.

The subgroup analyses presented by the company with the dossier were not used in dossier assessment A23-79 due to missing and partly discrepant data. In its comments, the company did not address the points of criticism listed in dossier assessment A23-79. Rather, the company proceeded analogously in its subgroup analyses subsequently submitted with the comments for the outcomes recorded using R-PAct and PROMIS (responder analyses at Week 52 based on transformed values), so that corresponding subgroup analyses for the characteristic “age” are also completely missing here and the model used by the company for interaction testing remains unclear.

Overall, due to the uncertainties mentioned in dossier assessment A23-79 that still exist after the commenting procedure, the subgroup analyses of the company were not used for the benefit assessment.

2.2 Probability and extent of added benefit

2.2.1 Assessment of added benefit at outcome level

The extent of the respective added benefit at outcome level was estimated from the results presented in dossier assessment A23-79 and the previous sections (see Table 3).

Table 3: Extent of added benefit at outcome level: cipaglucosidase alfa + miglustat vs. alglucosidase alfa (multipage table)

Outcome category **outcome**	**Cipaglucosidase alfa + miglustat vs. alglucosidase alfa + placebo** **event rate (%) or change at Week 52 (mean)** **effect estimation [95% CI];** **p-value** **probability**[a]	**Derivation of extent**[b]
Mortality		
All-cause mortality	0% vs. 0% RR: –[c]	Lesser/added benefit not proven
Morbidity (at Week 52)		
Physical endurance		
6MWT [metres]	21.44 vs. 16.11 MD: 5.33 [-15.21; 25.88] p = 0.608	Lesser/added benefit not proven
Motor function		
GSGC total value	-0.56 vs. 0.74 MD: -1.30 [-2.34; -0.26] p = 0.015 SMD: -0.51 [-0.94; -0.08][d]	Lesser/added benefit not proven
Physical functioning		
R-PAct (worsening)	1% vs. 0% RR: –[c]	Lesser/added benefit not proven
PROMIS Physical Function (worsening)	0% vs. 3% RR: –[c]	Lesser/added benefit not proven
Fatigue (PROMIS Fatigue; worsening)	6% vs. 8% RR: 0.78 [0.18; 3.39] p = 0.739	Lesser/added benefit not proven
Dyspnoea (PROMIS Dyspnea Severity; worsening)	No suitable data	Lesser/added benefit not proven
Function of the upper extremities (PROMIS Upper Extremity; worsening)	5% vs. 0% RR: 1.41 [0.36; 5.54] p = 0.618	Lesser/added benefit not proven
General physical well-being (SGIC; worsening)	18% vs. 29% RR: 0.65 [0.33; 1.26] p = 0.199	Lesser/added benefit not proven
Respiratory effort (SGIC; worsening)	8 % vs. 11 % RR: 0.79 [0.23; 2.75] p = 0.715	Lesser/added benefit not proven
Muscle strength (SGIC; worsening)	18% vs. 29% RR: 0.65 [0.34; 1.25] p = 0.195	Lesser/added benefit not proven

Table 3: Extent of added benefit at outcome level: cipaglucosidase alfa + miglustat vs. alglucosidase alfa (multipage table)

Outcome category **outcome**	**Cipaglucosidase alfa + miglustat vs. alglucosidase alfa + placebo** **event rate (%) or change at Week 52 (mean)** **effect estimation [95% CI];** **p-value** **probability**[a]	**Derivation of extent**[b]
Muscle function (SGIC; worsening)	14% vs. 29% RR: 0.50 [0.25; 1.02] p = 0.057	Lesser/added benefit not proven
Ability to move (SGIC; worsening)	11% vs. 34% RR: 0.32 [0.15; 0.67] p = 0.002 Probability: indication	Outcome category: non-serious/non-severe symptoms/late complications $CI_u < 0.80$ Added benefit; extent: "considerable"
Activities of daily living (SGIC; worsening)	9% vs. 13% RR: 0.82 [0.28; 2.41] p = 0.714	Lesser/added benefit not proven
Energy level (SGIC; worsening)	11% vs. 24% RR: 0.40 [0.18; 0.88] p = 0.023 Probability: indication	Outcome category: non-serious/non-severe symptoms/late complications $0.80 \leq CI_u < 0.90$ Added benefit; extent: "minor"
Muscle pain (SGIC; worsening)	19% vs. 24% RR: 0.78 [0.37; 1.66] p = 0.515	Lesser/added benefit not proven
Health status (EQ-5D VAS)	0.05 vs. 3.87 MD: -3.82 [-9.51; 1.87] p = 0.187	Lesser/added benefit not proven
Health-related quality of life		
Outcomes from this category were not recorded		
Side effects		
SAEs	9% vs. 3% RR: 3.58 [0.50; 25.61] p = 0.205	Greater/lesser harm not proven
Discontinuation due to AEs	2% vs. 3% RR: 0.86 [0.09; 8.63] p = 0.898	Greater/lesser harm not proven
Infusion-related reactions (AEs)	25% vs. 26% RR: 0.91 [0.48; 1.72] p = 0.770	Greater/lesser harm not proven
Infusion-related reactions (SAEs)	1% vs. 0% RR: –[c]	Greater/lesser harm not proven

Table 3: Extent of added benefit at outcome level: cipaglucosidase alfa + miglustat vs. alglucosidase alfa (multipage table)

Outcome category **outcome**	**Cipaglucosidase alfa + miglustat vs. alglucosidase alfa + placebo** **event rate (%) or change at Week 52 (mean)** **effect estimation [95% CI];** **p-value** **probability[a]**	**Derivation of extent[b]**
a. Probability provided there is a statistically significant and relevant effect. b. Depending on the outcome category, estimations of effect size and the scale of the outcome are made with different limits based on the upper or lower limit of the confidence interval (CI_u or CI_L). c. No presentation of the effect estimation, as no person in one treatment arm and at most 1 person in the other treatment arm had an event. d. If the CI for the SMD is fully outside the irrelevance range [-0.2; 0.2], this is interpreted to be a relevant effect. In other cases, the presence of a relevant effect cannot be derived. 6MWT: 6-minute walk test; AE: adverse event; CI: confidence interval; CI_L: lower limit of confidence interval; CI_u: upper limit of confidence interval; GSGC: Gait, Stairs, Gower's manoeuvre, Chair; MD: mean difference; PROMIS: Patient Reported Outcome Measurement Information System; R-PAct: Rasch-built Pompe-specific Activity; RR: relative risk; SAE: serious adverse event; SGIC: Subject's Global Impression of Change; SMD: standardized mean difference; VAS: visual analogue scale		

2.2.2 Overall conclusion on added benefit

Table 4 summarizes the results of dossier assessment A23-79 [1] and the present addendum A23-133, which were considered for the overall conclusion on the extent of added benefit.

Table 4: Positive and negative effects from the assessment of cipaglucosidase alfa + miglustat in comparison with alglucosidase alfa

Positive effects	**Negative effects**
Non-serious/non-severe symptoms/late complications ▪ ability to move (SGIC), worsening: indication of an added benefit – extent: "considerable" ▪ energy level (SGIC): worsening: indication of an added benefit – extent: "minor"	–
Outcomes on health-related quality of life were not recorded.	
SGIC: Subject's Global Impression of Change	

In the PROPEL study used for the benefit assessment, the patients' symptoms were recorded using numerous patient-reported outcomes (14 instruments) in addition to various functional tests. For two of these outcomes, there were positive effects of cipaglucosidase alfa + miglustat compared to alglucosidase alfa: For "ability to move" and "energy level", there was an indication of an added benefit, with the extent "considerable" or "minor". Each of these

outcomes was recorded using individual questions (Subject's Global Impression of Change [SGIC]). Data on the instruments PROMIS and R-PAct were subsequently submitted in the commenting procedure. Especially the PROMIS instruments comprehensively represent the symptoms. The results on the outcomes recorded using PROMIS and R-PAct do not confirm the positive effects observed, however, they do not fundamentally call them into question. Outcomes on health-related quality of life were not recorded.

In summary, there is a hint of a minor added benefit of cipaglucosidase alfa + miglustat over the ACT alglucosidase alfa for adult patients with LOPD in this data constellation.

2.3 Summary

The data subsequently submitted by the company in the commenting procedure change the conclusion on the added benefit of cipaglucosidase alfa + miglustat from dossier assessment A23-79: For adult patients with LOPD, there is an indication of a minor added benefit.

The following Table 5 shows the result of the benefit assessment of cipaglucosidase alfa + miglustat under consideration of dossier assessment A23-79 and the present addendum.

Table 5: Cipaglucosidase alfa + miglustat – probability and extent of added benefit

Therapeutic indication	**ACT[a]**	**Probability and extent of added benefit**
Adults with LOPD (GAA deficiency)	Alglucosidase alfa[b]	Indication of minor added benefit[c]

a. Presented is the respective ACT specified by the G-BA.
b. If indicated, physiotherapy measures should be made available to patients in both arms of the study.
c. The PROPEL study included patients with seated FVC ≥ 30% who achieved ≥ 75 m and ≤ 90% of the predicted value for healthy adults in the 6MWT and did not require invasive or non-invasive respiratory support for > 6 hours per day while awake (see dossier assessment A23-79 [1]). It remains unclear whether the effects observed in the study are transferable to patients with severe impairment of lung function and endurance.

6MWT: 6-minute walk test; FVC: forced vital capacity; GAA: acid α-glucosidase; G-BA: Federal Joint Committee; LOPD: late-onset Pompe disease

The G-BA decides on the added benefit.

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