Gemeinsamer
Bundesausschuss

Gemeinsamer Bundesausschuss

BBS-Webinar: **HTA-Perspective on the assessment of CAR-T-Cell Therapies**

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Brief overview of reimbursement in Germany

- After market access, the pharmaceutical company needs to submit a dossier to show additional benefit as compared to an appropriate comparator defined by the Federal Joint Committee (G-BA: Gemeinsamer Bundesausschuss)
- The G-BA assesses recognition of any additional benefit of a new drug

  → *Benefit assessments are usually delegated to IQWiG (Institute for Quality and Efficiency in Health Care)*
- G-BA publishes a resolution based on benefit assessment and hearings with a decision on the pricing procedure for the new medicine

  → **Additional benefit proven: Negotiation of a new price**

  → **Otherwise: Categorisation to pricing of the comparative therapy**

Orphan drugs at G-BA

Additional medicinal benefit is already assumed proven by market authorization, but the extent of additional benefit needs to be assessed

Two pathways:

1. Below turnover of 50 Mio € per year

   → **Restricted benefit assessment (G-BA)**

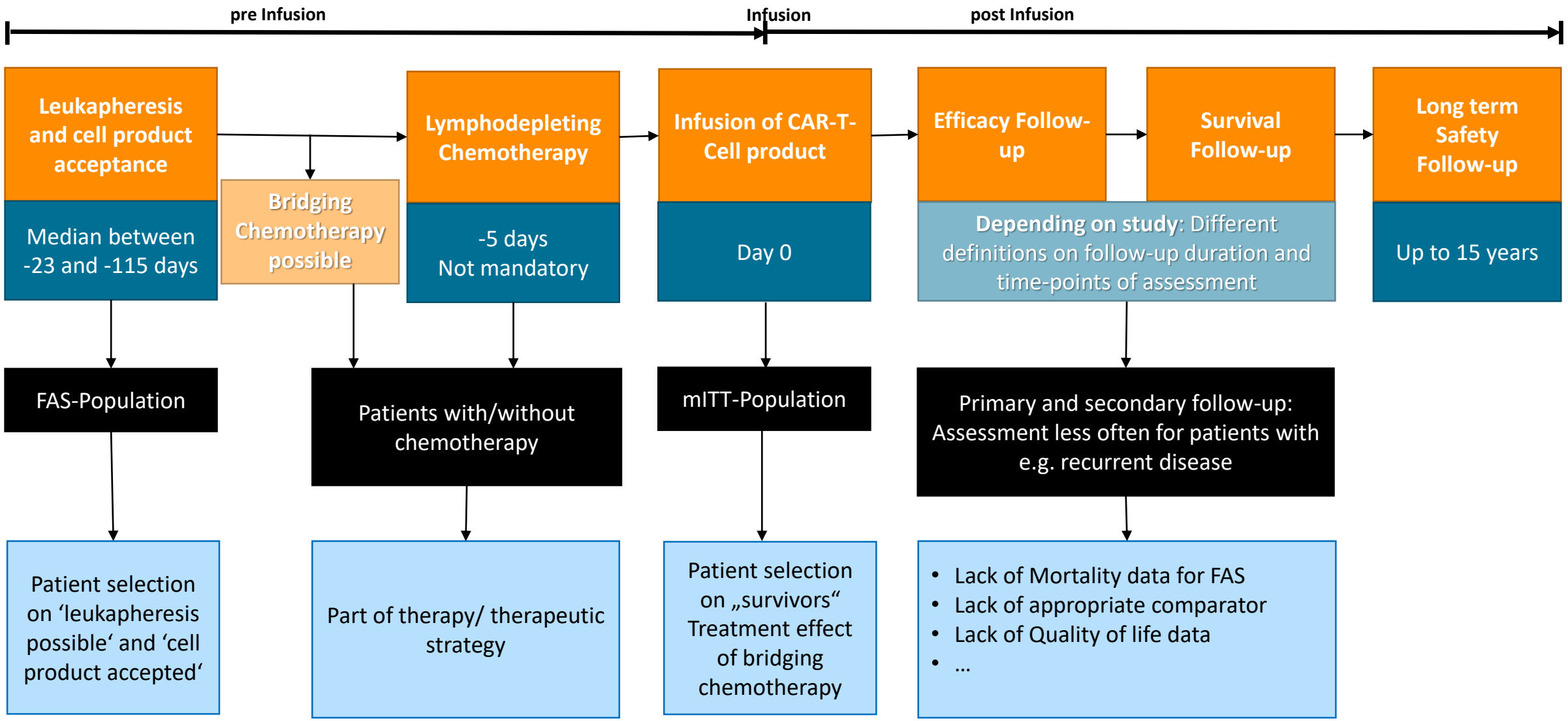
2. Above turnover of 50 Mio € per year

   → **Like non-orphan drugs: Additional benefit and its extent as compared to an appropriate comparator (IQWiG)**

CAR-T-Cell Therapy assessment at G-BA

	Axicabtagen-Ciloleucel	Tisagenlecleucel-DLBCL	Tisagenlecleucel-ALL
Population	Treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) and primary mediastinal large B-cell lymphoma (PMBCL), after two or more lines of systemic therapy	Treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) after two or more lines of systemic therapy	Treatment of paediatric and young adult patients up to and including 25 years of age with B-cell acute lymphoblastic leukaemia (ALL) that is refractory, in relapse post-transplant or in second or later relapse
Timelines	1st resolution 2019 (limited to 2022)	1st resolution 2019 (limited to 2020) 2nd resolution 2020 (limited to 2023)	1st resolution 2019 (limited to 2020) 2nd resolution 2020 (limited to 2023)
Extent of additional benefit	Non-quantifiable	Hint for a non-quantifiable additional benefit because the scientific data does not permit quantification	Hint for a non-quantifiable additional benefit because the scientific data does not permit quantification

(Simplified) conduct of studies assessed at G-BA[1)2)]



[1)]Not all points apply to all studies assessed by G-BA
[2)]Not all critical points are listed here

Summary

- **P**opulation: Definition of target population vs. analysis population
- **I**ntervention: Definition of CAR-T-cell therapy/ therapeutic strategy
- **C**omparator: Appropriate comparator for quantification of added benefit
- **O**utcome: Mortality, Morbidity (patient relevant endpoints), Quality of Life, Safety
- **S**tudy design: RCTs preferable

References

Gemeinsamer Bundesausschuss (G-BA). Justification to the Resolution of the Federal Joint Committee (G-BA) on an amendment to the Pharmaceuticals Directive (AM-RL): Annex XII – Resolutions on the benefit assessment of medicinal products with new active ingredients according to Section 35a SGB – Axicabtagene ciloleucel from 2 May 2019. Berlin (GER): G-BA; 2019. [Zugriff: 15.03.2021]. URL: https://www.g-ba.de/downloads/40-1465-5741/2019-05-02_AM-RL-XII_Axicabtagen-Ciloleucel_D-406_D-416_TrG_EN.pdf.

Gemeinsamer Bundesausschuss (G-BA). Justification to the Resolution of the Federal Joint Committee (G-BA) on an Amendment of the Pharmaceuticals Directive (AM-RL): Annex XII – Benefit Assessment of Medicinal Products with New Active Ingredients According to Section 35a SGB V – Tisagenlecleucel (Reassessment after Expiry: Diffuse Large B-cell Lymphoma) from 17 September 2020. Berlin (GER): G-BA; 2020. [Zugriff: 15.03.2021]. URL: https://www.g-ba.de/downloads/40-1465-6853/2020-09-17_AM-RL-XII_Tisagenlecleucel-DLBCL_D-530_TrG_EN.pdf.

Gemeinsamer Bundesausschuss (G-BA). Justification to the Resolution of the Federal Joint Committee (G-BA) on an Amendment of the Pharmaceuticals Directive (AM-RL): Annex XII – Benefit Assessment of Medicinal Products with New Active Ingredients According to Section 35a SGB V – Tisagenlecleucel (Reassessment after Expiry: B cell Acute Lymphoblastic Leukaemia) from 17 September 2020. Berlin (GER): G-BA; 2020. [Zugriff: 15.03.2021]. URL: https://www.g-ba.de/downloads/40-1465-6854/2020-09-17_AM-RL-XII_Tisagenlecleucel_D-529_TrG_EN.pdf.

Thank you for your attention!