Comprehensive Cancer Center
Tübingen-Stuttgart

Post ASH 2023 San Diego

Hämostaseologie

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Fokus Immunthrombozytopenie (ITP)

ITP 1: Avatrombopag bei früher versus bei chronischer ITP

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Results: Platelet Outcomes

Outcome	Newly Dx/ Persistent ITP N = 23	Chronic ITP N = 52	*P* value
Response, n (%)	19 (91)	46 (96)	0.58
Response fraction, mean (95% CI)	0.74 (0.59-0.89)	0.84 (0.76-0.92)	0.16
Complete response, n (%)	18 (86)	39 (81)	0.78
Complete response fraction, mean (95% CI)	0.54 (0.36-0.72)	0.66 (0.55-0.78)	0.26

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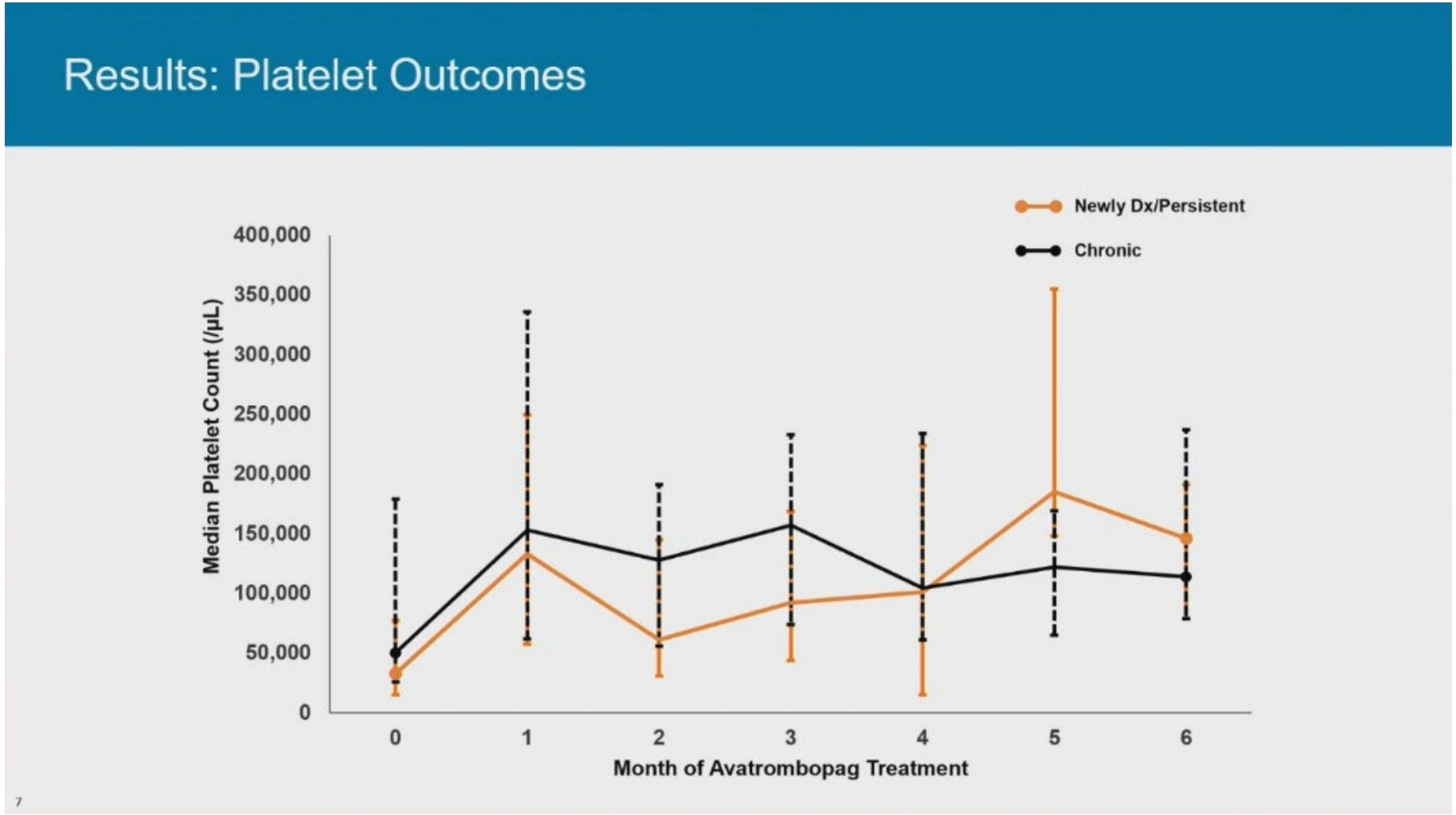
ITP 1: Avatrombopag bei früher versus bei chronischer ITP



ITP 2: Romiplostim initial hochdosiert vs. niedrigdosiert

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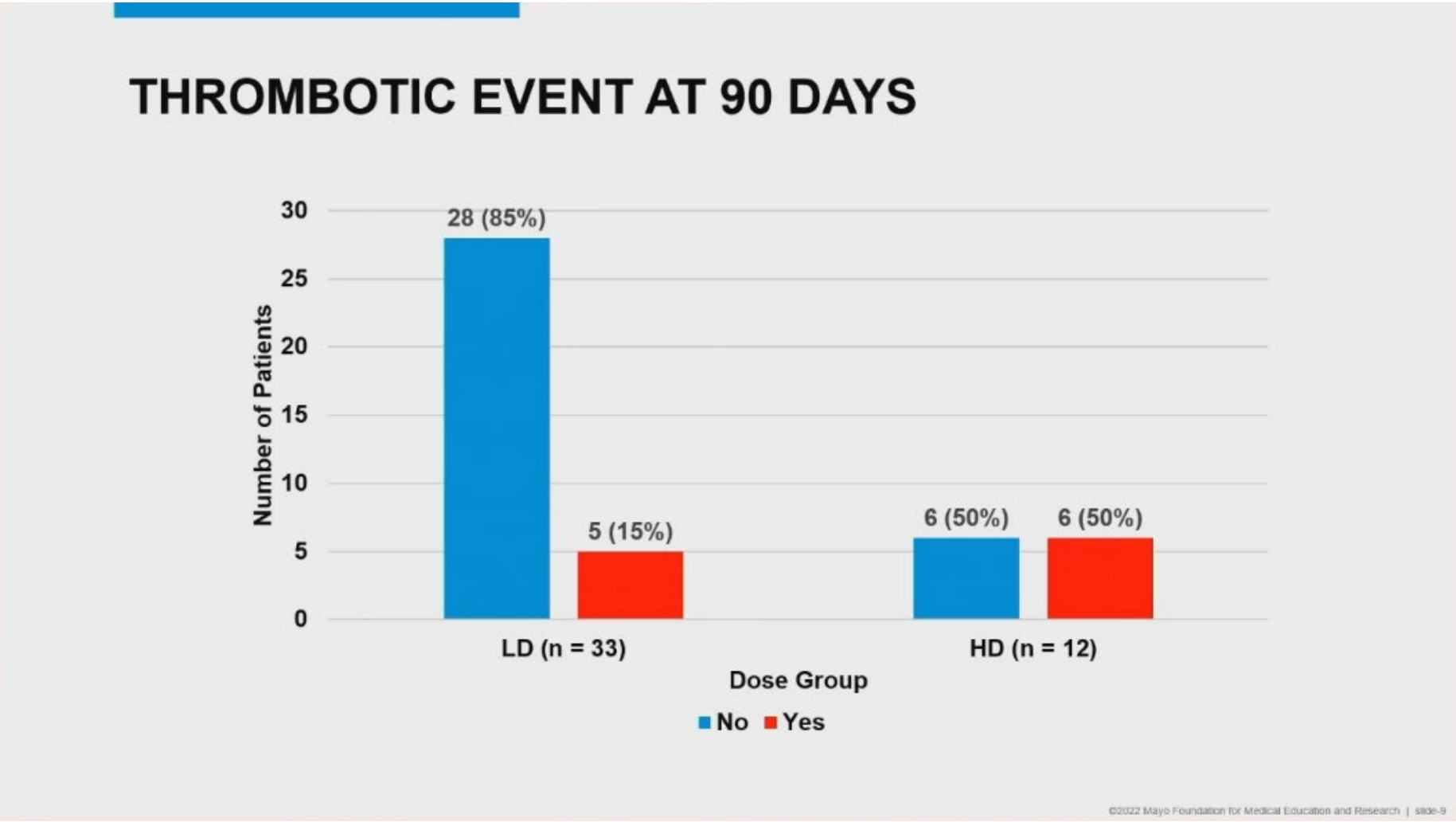

EfPatient Outcomes after Initial High Dose Versus Low Dose Romiplostim for Inpatient Management of Immune Thrombocytopenia by Elizabeth King

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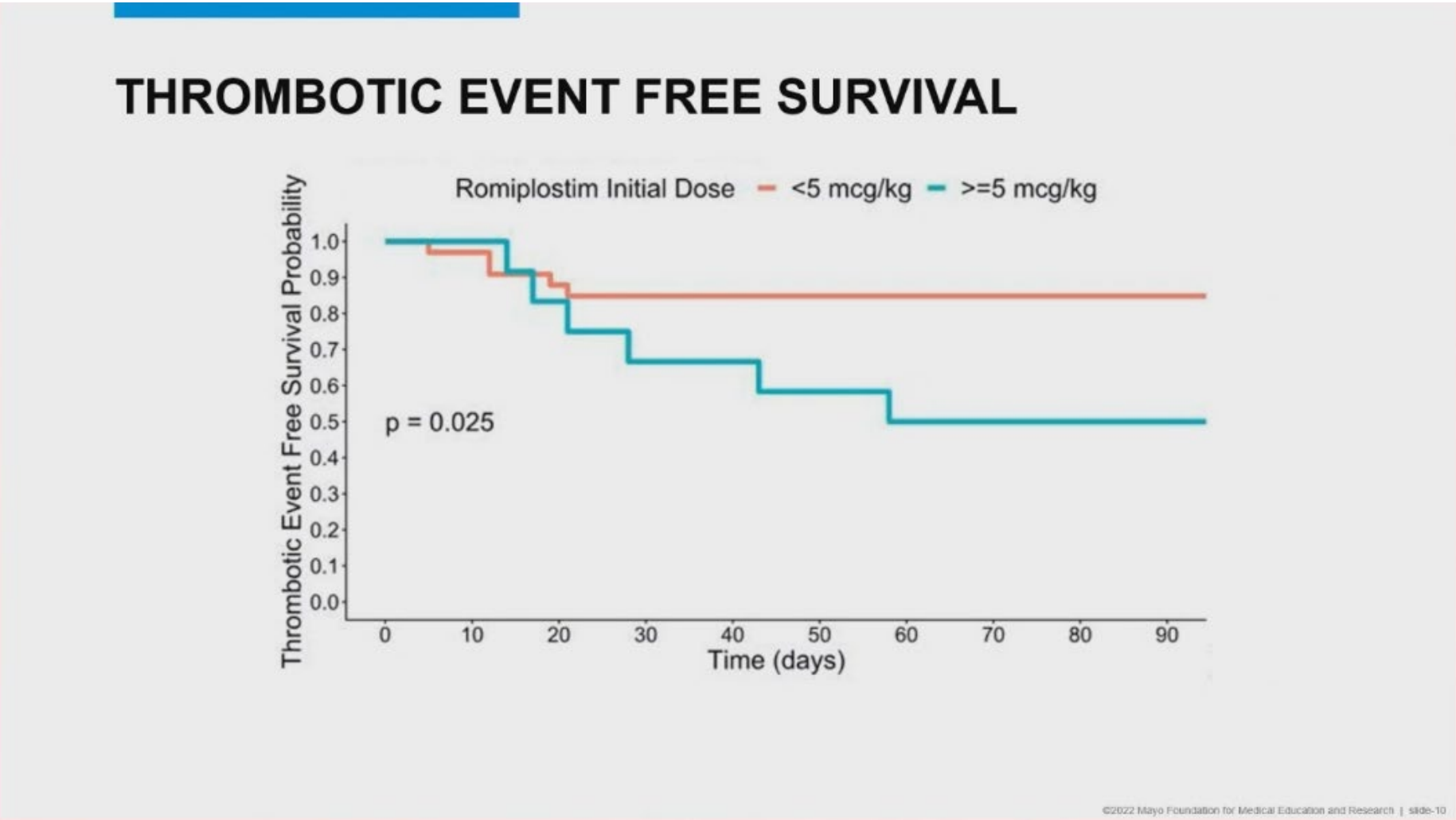

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ITP 3: Risiko von Infektionen bei ITP

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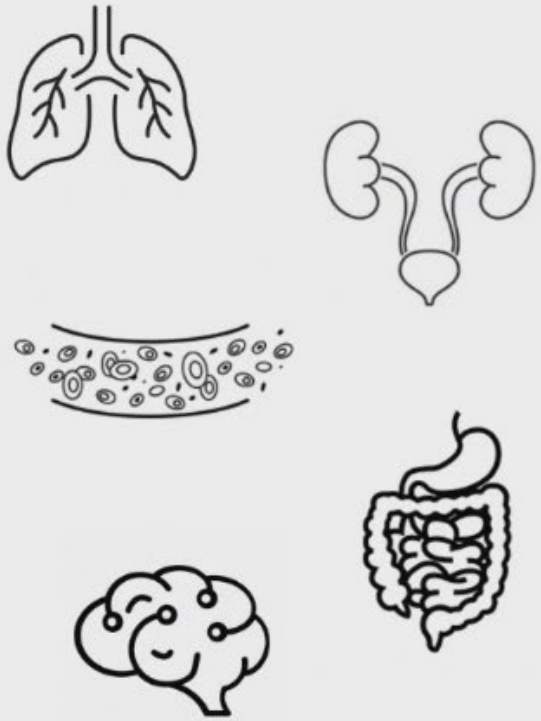
Subgroups of infection

7 groups of infections

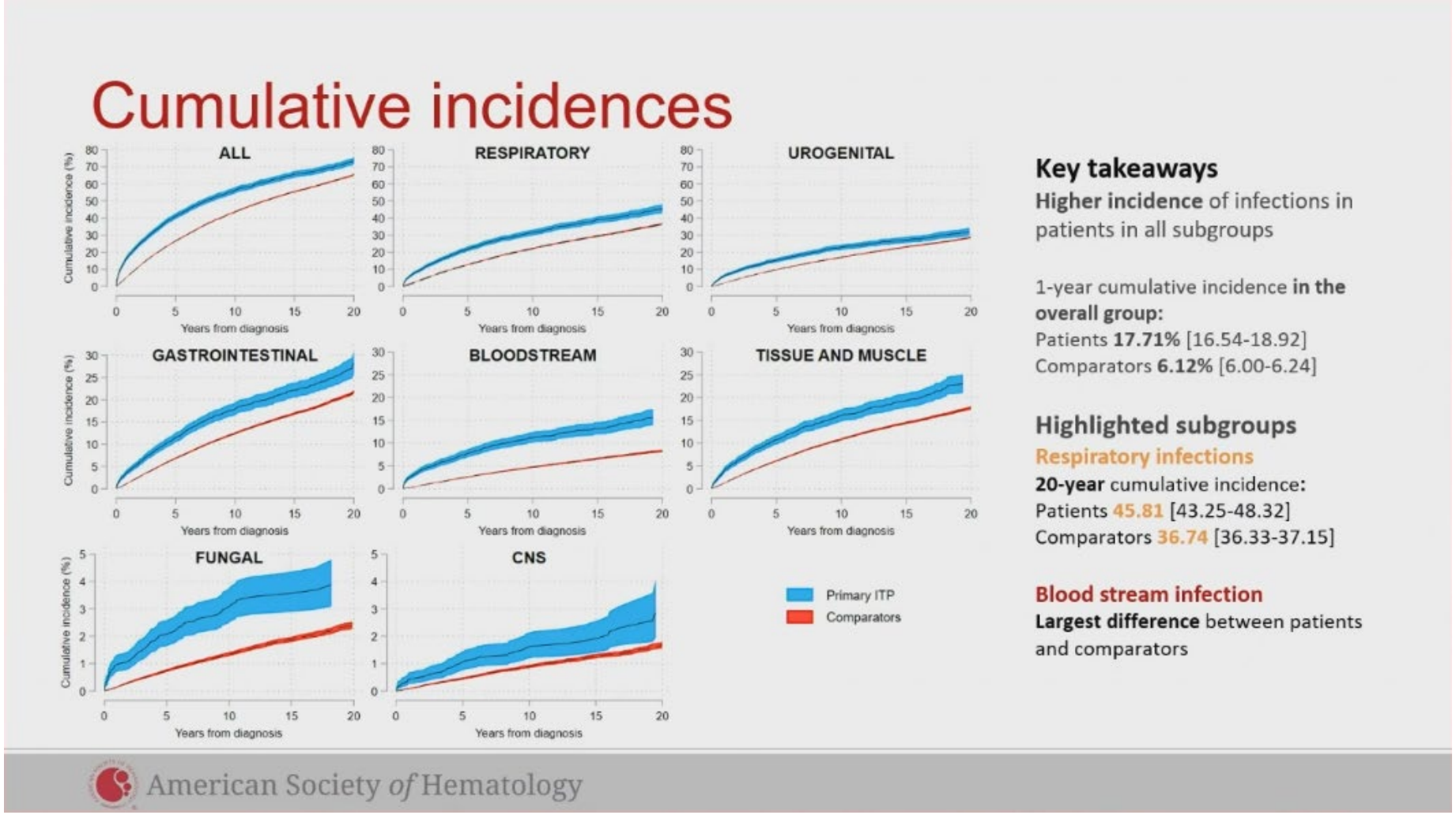
1. Respiratory
2. Urogenital
3. Bloodstream
4. Gastrointestinal
5. Tissue and musculoskeletal
6. CNS
7. Candidiasis and other fungal

All (combined group of the 7 subgroups)
Defined as the first of any type of infections



American Society *of* Hematology

ITP 3: Risiko von Infektionen bei ITP



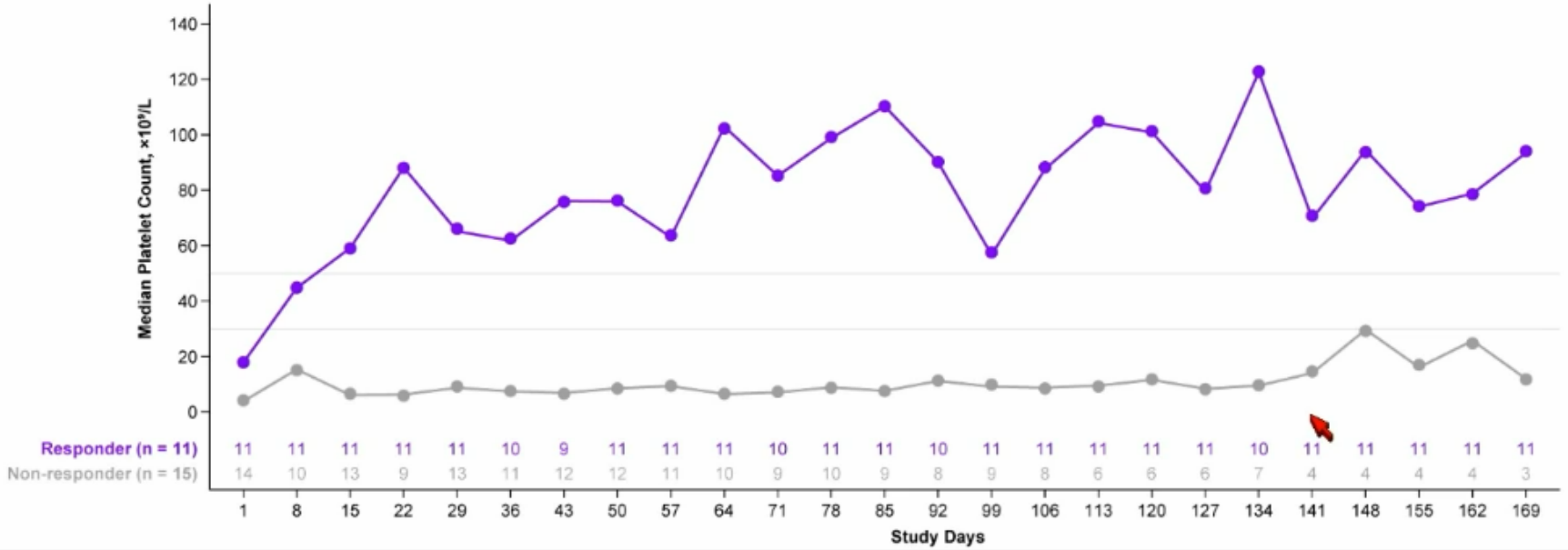
ITP 4: BTK-Inhibitor Rilzabrutinib bei rezidivierter ITP

Initial Report of Part B Phase 1/2 Efficacy and Safety Results for Bruton Tyrosine Kinase Inhibitor Rilzabrutinib in Patients with Relapsed Immune Thrombocytopenia by Nichola Cooper

ITP 4: BTK-Inhibitor Rilzabrutinib bei rezidivierter ITP

Efficacy: Responder and Non-Responder

- 9 (35%) patients (95% CI, 17%, 56%) achieved primary endpoint of durable response
  - Durable response: platelet counts ≥50×10^9/L on ≥8 of the last 12 weeks without rescue medication
- 11 (42%) Responder patients continued in the LTE period (n = 9 met primary endpoint + n = 2 LTE eligible)
  - LTE eligibility: Platelet counts ≥50 ×10^9/L or ≥30 ×10^9/L and doubling of baseline for ≥4 of last 8 weeks without rescue medication



American Society of Hematology Data cutoff: 31 Jan 2023. 7

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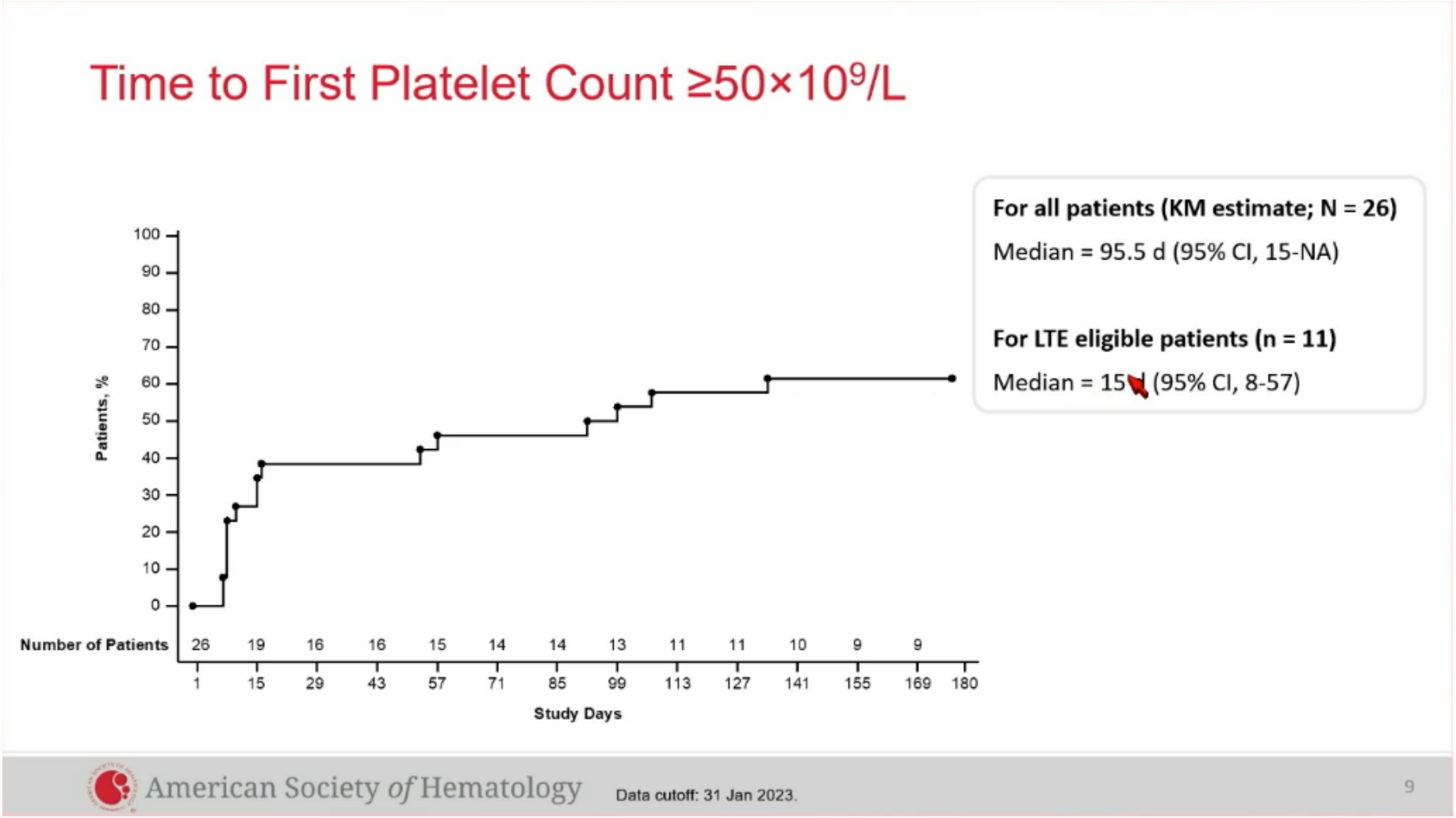

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ITP 5: Efgartigimod bei ITP

Time to Achieve Platelet Count Response after Intravenous Efgartigimod in Adults with Primary Immune Thrombocytopenia: A Phase 3, Multicenter, Double-Blinded, Placebo-Controlled, Randomized Clinical Trial (ADVANCE IV) by Catherine Broome

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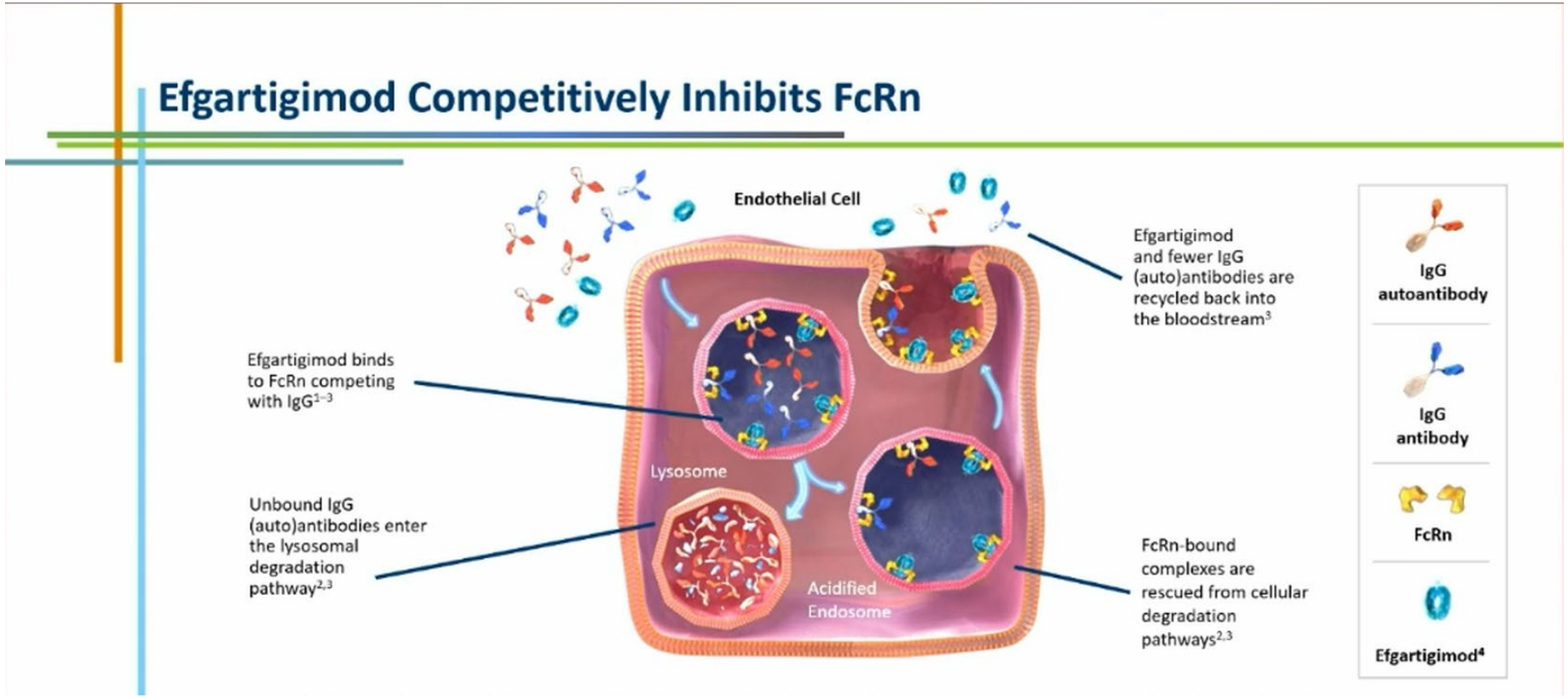

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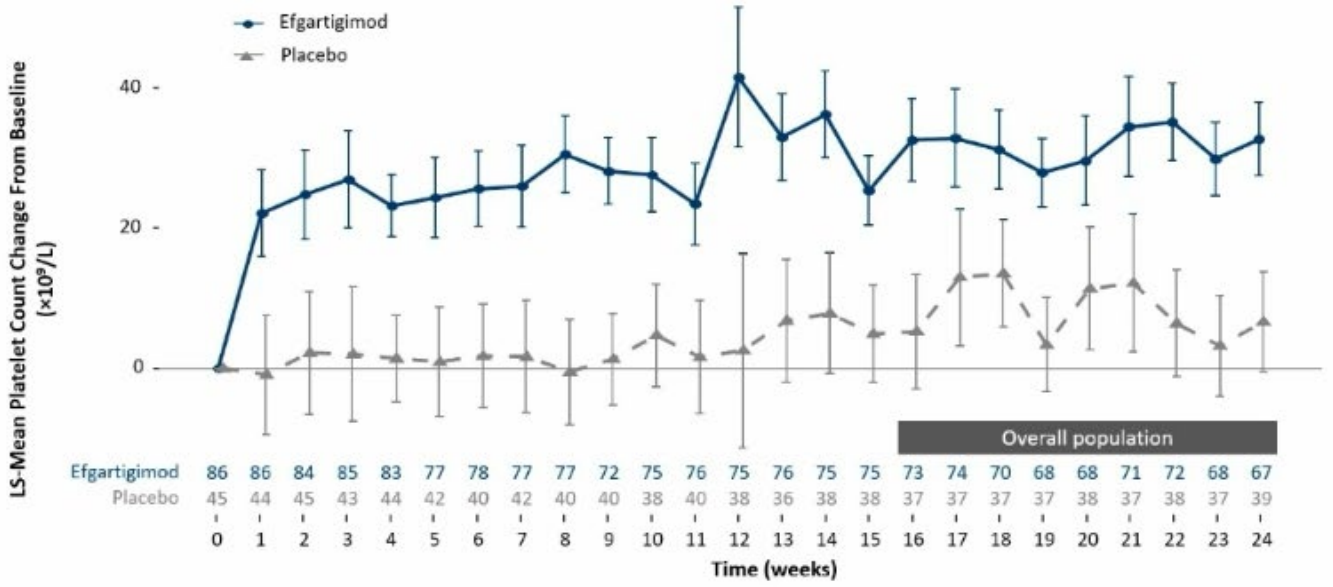
ITP 5: Efgartigimod bei ITP

Significant Platelet Count Increases Compared With Placebo Were Seen by Week 1[1]



- **38.4% (n=33)** of efgartigimod-treated participants compared with **11.1% (n=5)** of placebo-treated participants achieved a platelet count **of ≥30×10⁹/L** in 1 week[1]

LS, least squares.
1. Broome CM, et al. *Lancet*. 2023;402:1648–59.

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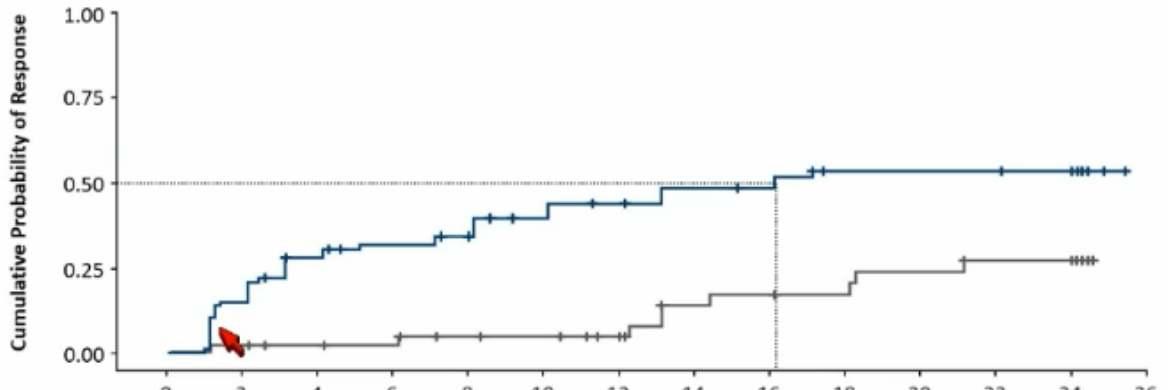
ITP 5: Efgartigimod bei ITP

A Greater Proportion of Participants in the EFG Group Had a Platelet Count Response by Week 6 Compared With Those in the PBO Group

Time to Platelet Count Response (2 Consecutive Platelet Counts of ≥50×10⁹/L)



Number at risk														
Efgartigimod	86	73	58	53	50	42	38	33	32	27	27	27	26	0
Placebo	45	44	42	41	38	37	34	27	26	25	23	21	21	0

- Based on KM analysis, **31.8%** of participants receiving efgartigimod and **2.2%** of those receiving placebo achieved platelet count responses by Week 6

EFG, efgartigimod; KM, Kaplan-Meier; PBO, placebo.
Dashed black line denotes 50% response in the EFG arm.

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ITP 6: CD19 CAR-T-Zellen bei ITP

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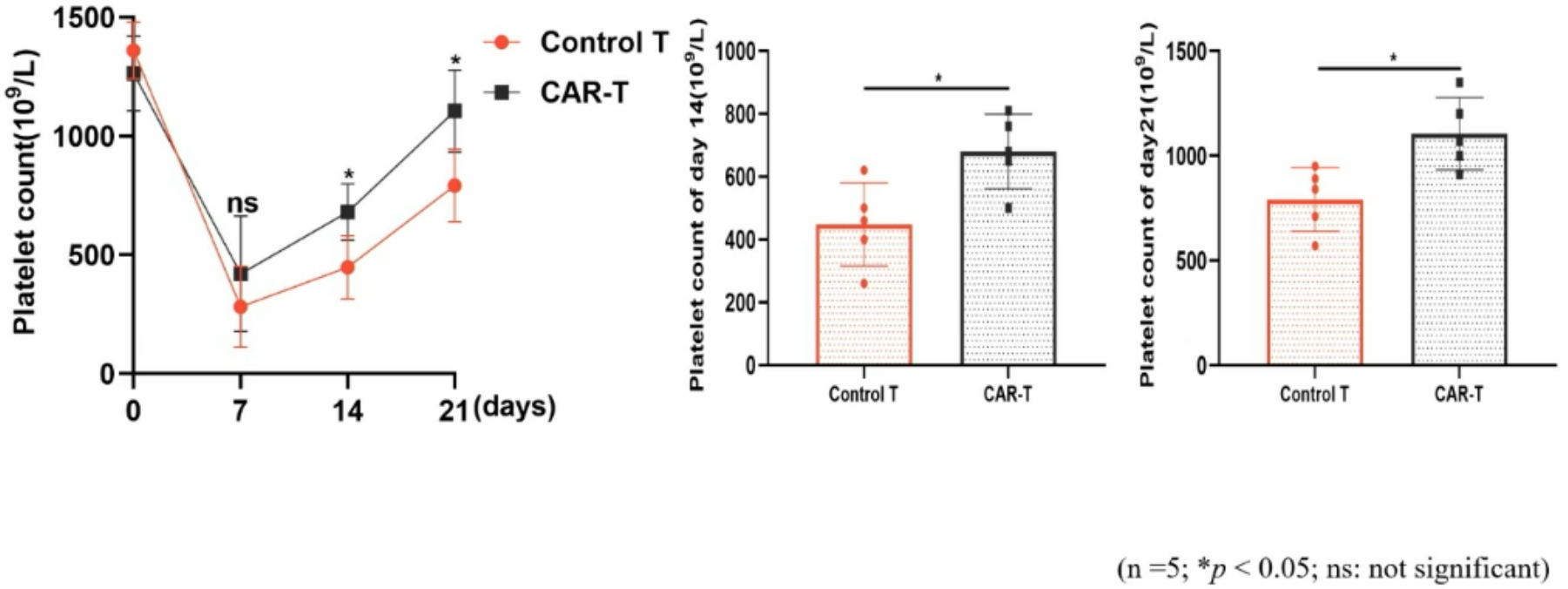
Results

- **Platelet count**

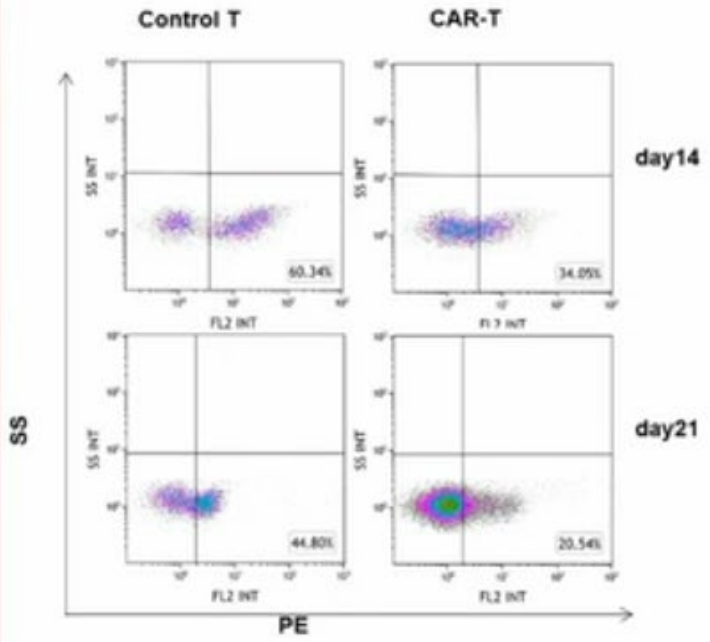


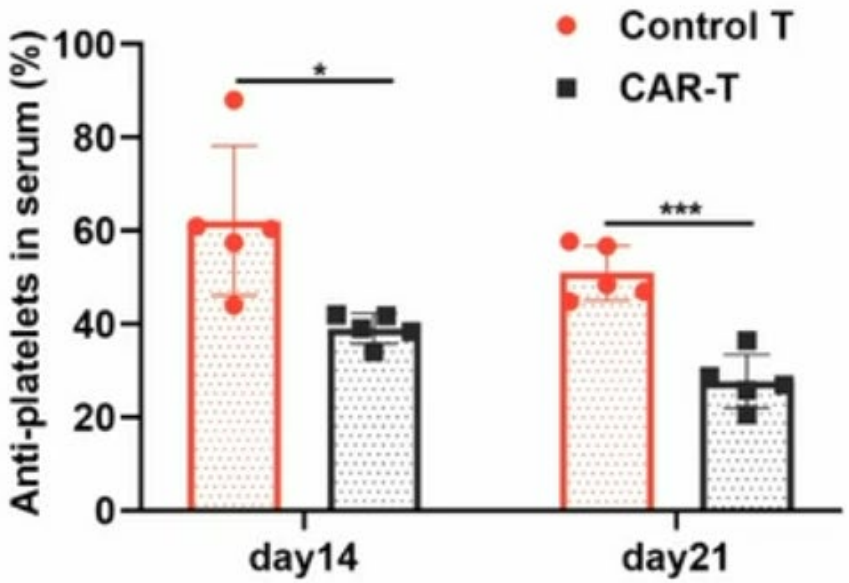
(n =5; *$p < 0.05$; ns: not significant)

ITP 6: CD19 CAR-T-Zellen bei ITP

Results

- **Anti-platelets in serum**



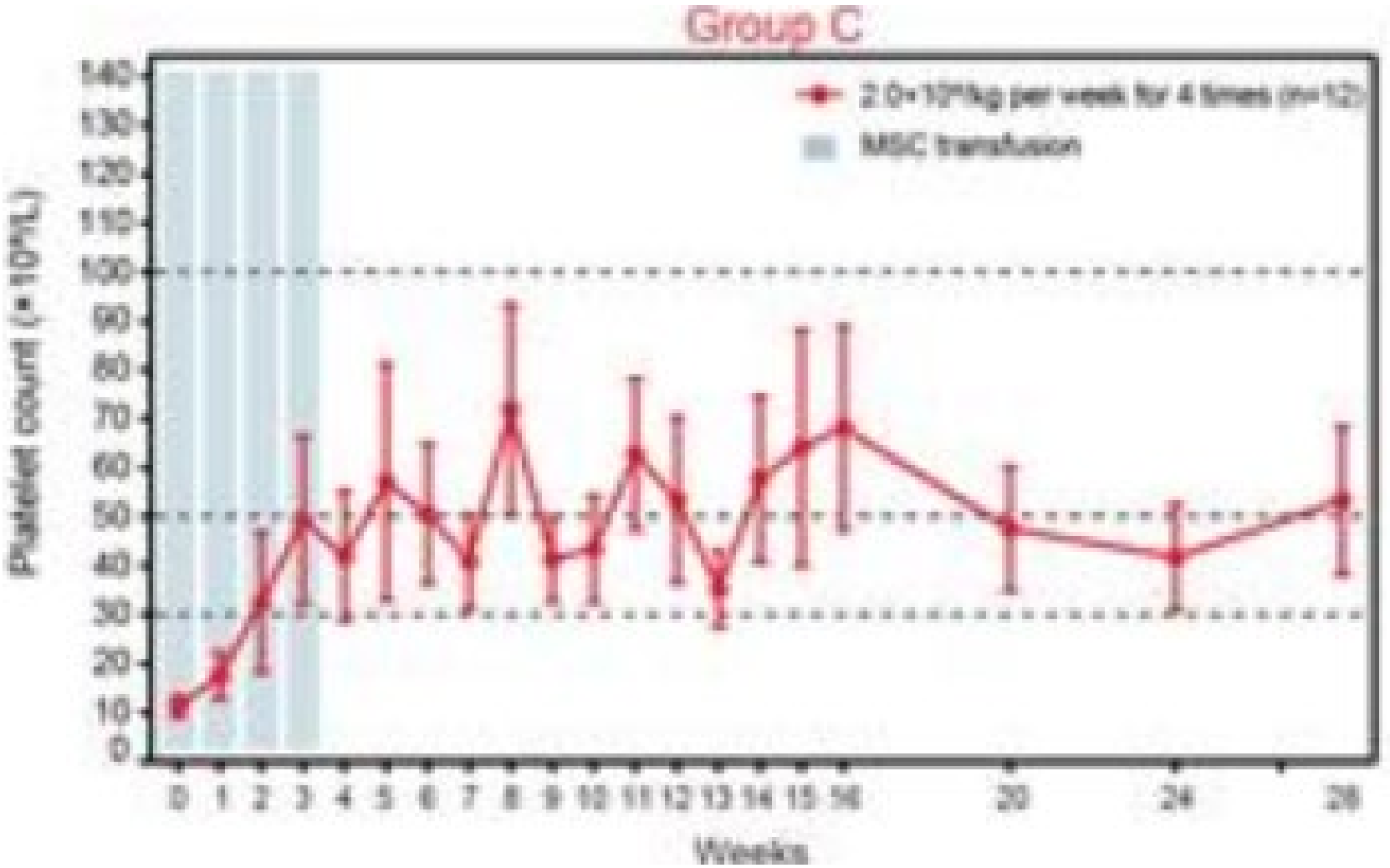


(*$p < 0.05$; ***$p < 0.001$; ns: not significant)

ITP 7: Mesenchymale Stromazellen bei ITP

Efficacy and Safety of Human Umbilical Cord-Derived Mesenchymal Stem Cells in the Treatment of Immune Thrombocytopenia: A Prospective, Single Centre, Single Arm, Phase I Trial by Xu Yanmei

ITP 7: Mesenchymale Stromazellen bei ITP



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und am Ende….

Vielen Dank für Ihre Aufmerksamkeit

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