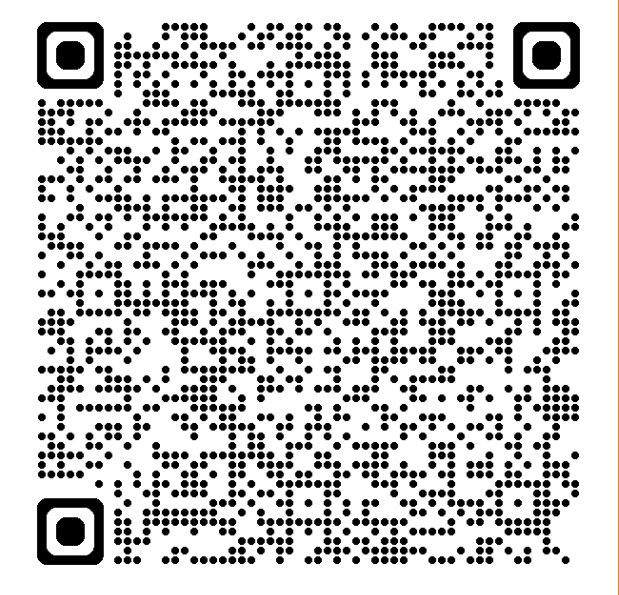


Pre- and Post-Transplant Survival with Emapalumab in Primary HLH: Pooled Analysis of Prospective Trials NI-0501-04, NI-0501-05 and NI-0501-09

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*At the time of analysis completion



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CONCLUSIONS

- **Interferon-γ (IFNγ) neutralization with emapalumab resulted in a high survival rate, effective bridging to hematopoietic stem-cell transplantation (HSCT), and favorable post-HSCT overall survival (OS) in primary hemophagocytic lymphohistiocytosis (pHLH) in this post-hoc analysis.**
- **Achieving a complete response (CR) or partial response (PR), based on investigator assessment, with emapalumab before conditioning was associated with a high post-transplant survival rate, reinforcing emapalumab as a validated bridge to curative HSCT.**

INTRODUCTION

- pHLH is a severe hyperinflammatory syndrome in which uncontrolled IFNγ production could result in multiorgan failure and death if not treated promptly and adequately.¹⁻³
- Allogeneic HSCT is the only potentially curative option for pHLH and requires prior control of hyperinflammation.^{2,4}
- Historically, low OS rates in pHLH were largely due to fatal complications during the pre-HSCT or immediate post-HSCT phases.⁵
- Emapalumab, a fully human immunoglobulin-G1 anti-IFNγ monoclonal antibody that binds both free and receptor-bound IFNγ, neutralizing its biological activity, is approved by the US Food and Drug Administration (FDA) for adults and children with pHLH with refractory, recurrent or progressive disease or intolerance to conventional HLH therapy.⁶
- The NI-0501-04 and NI-0501-09 studies demonstrated that emapalumab rapidly controls hyperinflammation, allowing conditioning for HSCT.^{7,8}
- This post-hoc analysis evaluates the pre- and post-transplant survival with emapalumab in pHLH based on the investigator’s global assessment of treatment response to closely emulate real-world clinical practice.

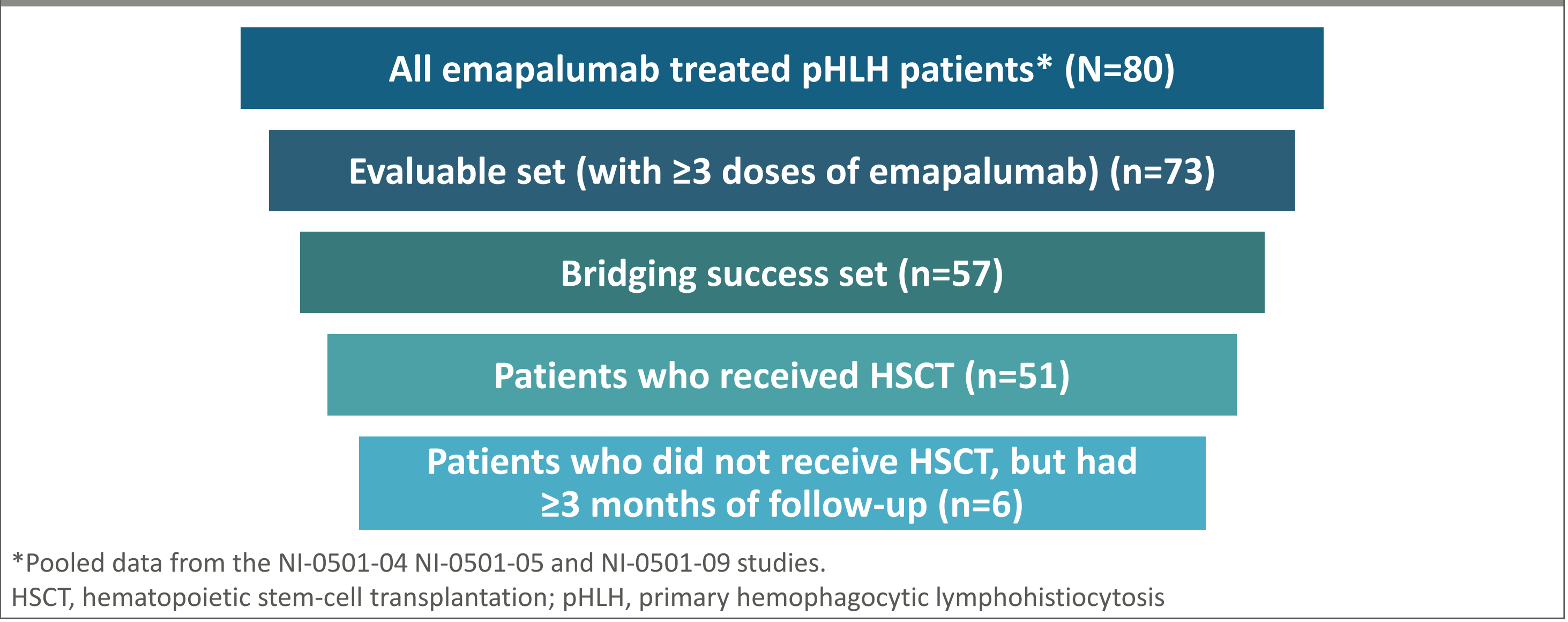
OBJECTIVE

- To conduct a pooled, post-hoc analysis of three prospective emapalumab trials, assessing (i) early on-therapy survival at Week 8, (ii) “bridging-success” survival, defined as survival to HSCT or alive ≥3 months post end-of-treatment (EOT) without HSCT, and (iii) OS post-HSCT

METHODS

- Individual patient data were pooled from studies NI-0501-04 (n=45),⁷ its long-term follow-up study NI-0501-05 (n=37 of 45 in NI-0501-04),⁸ and NI-0501-09 (n=35).⁹ A total of 80 patients from these studies were included (Figure 1).

Figure 1: Patients included in the post-hoc analyses



- Investigator’s global assessment was analyzed and classified as complete response (CR or non-active disease), partial response (PR or HLH improvement) or no response [NR] (progressive disease, reactivation, worsening or death), to more closely reflect real-world clinical practice. Investigator-assessed overall response (OR) was CR+PR.
- Safety has been previously reported.^{7,8}

RESULTS

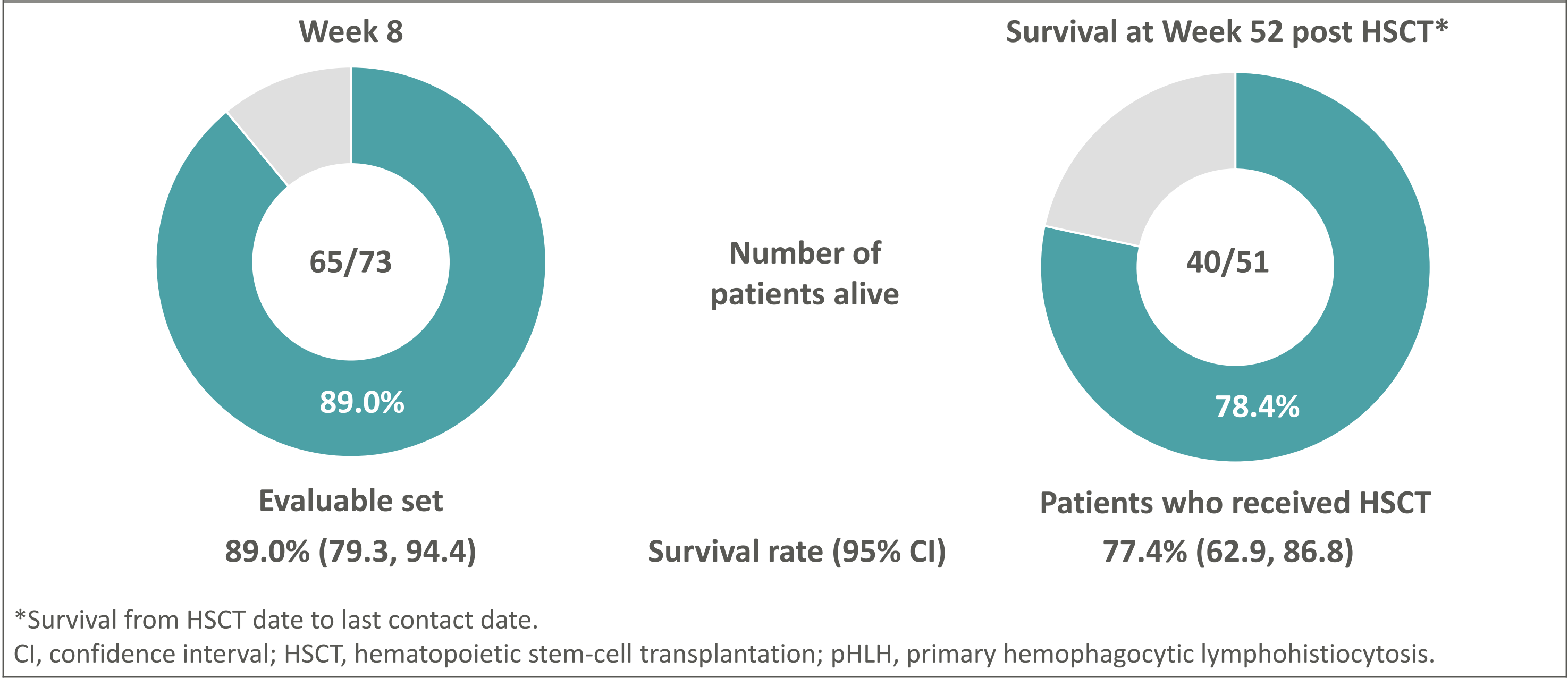
Patient Demographics and Baseline Characteristics

- Baseline median age was 0.8 years (range 0.01–16.67); 54% were male, 31% had central nervous system involvement, and 66% were previously treated with HLH therapy.

Survival at Weeks 8 and 52

- Treatment with emapalumab resulted in high survival rates at Weeks 8 and 52 in patients with pHLH (Figure 2).
- At the time of the analysis, median OS was not evaluable for either analysis set.

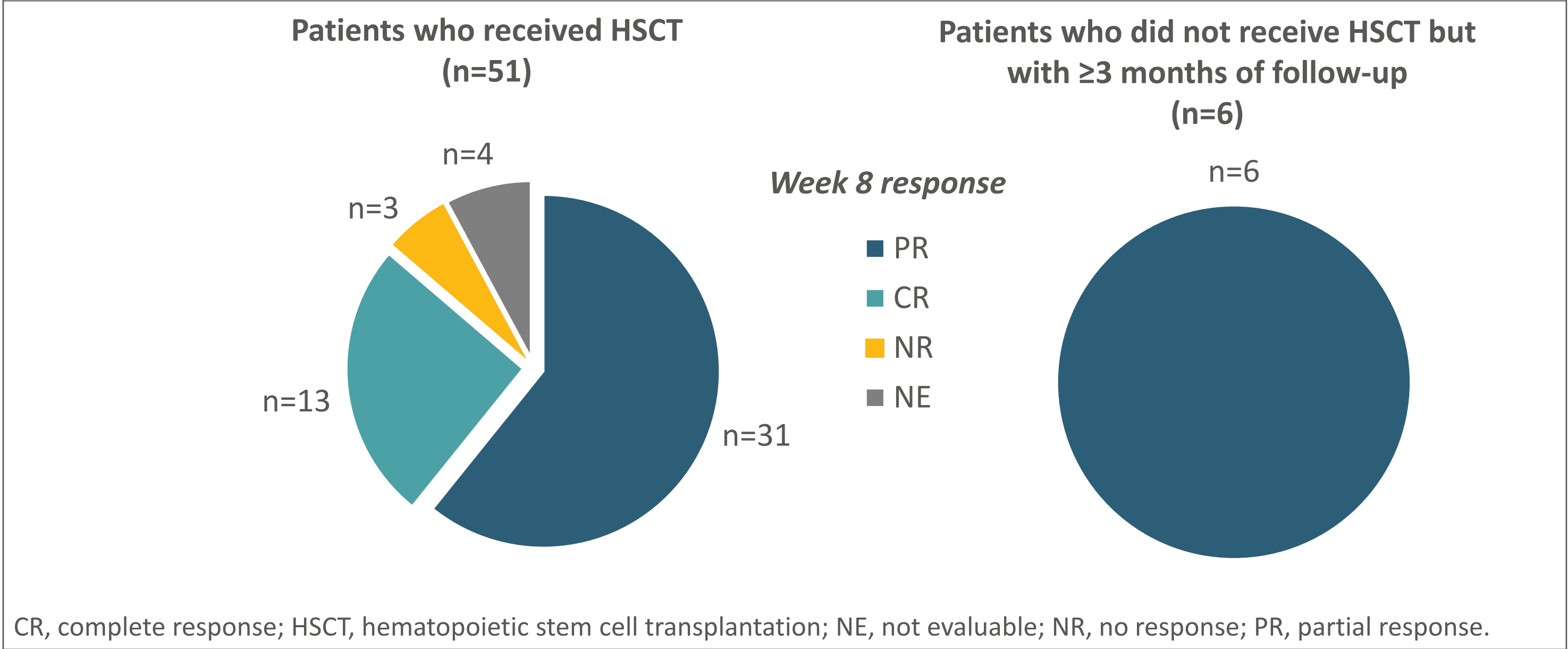
Figure 2: Emapalumab resulted in a high survival rate in patients with pHLH



Bridging-Success Survival

- Bridging-success survival was 78.1% (57/73) (Figure 3).
 - 51/57 (89.5%) proceeded to HSCT (median 358 days; [IQR: 106–385])
 - 6/57 (10.5%) without HSCT were alive ≥3 months after EOT (median 357 days; [IQR: 151–380])
- Investigator's Week 8 assessment showed that approximately seven times as many patients with bridging-success survival had an overall response (CR/PR) than NR/NE.

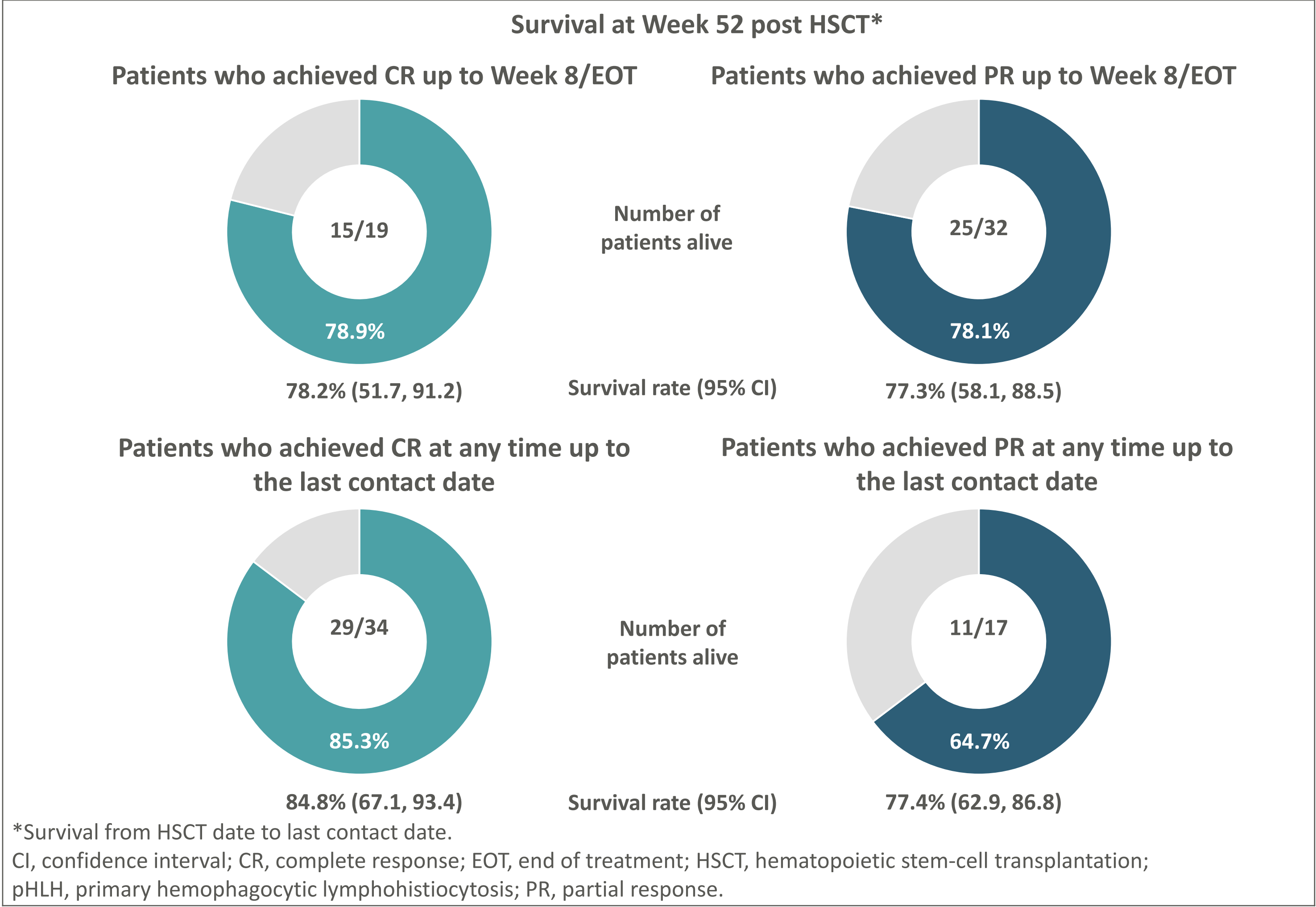
Figure 3: Most patients with bridging-success survival had CR or PR at Week 8/EOT as assessed by the investigator



Post-HSCT Survival

- Post-HSCT survival for patients showing CR/PR at Week 8/EOT was high (Figure 4)
 - 78.9% in patients who achieved investigator-assessed CR.
 - 78.1% in patients who achieved investigator-assessed PR.

Figure 4: High post-HSCT survival rates were observed in patients achieving investigator-assessed CR/PR



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