XIX CONGRESSO NAZIONALE SIES 2026

Minimal Residual Disease–Dependent Unfavorable Outcomes in Pediatric PAX5-Rearranged B-Acute Lymphoblastic Leukemia

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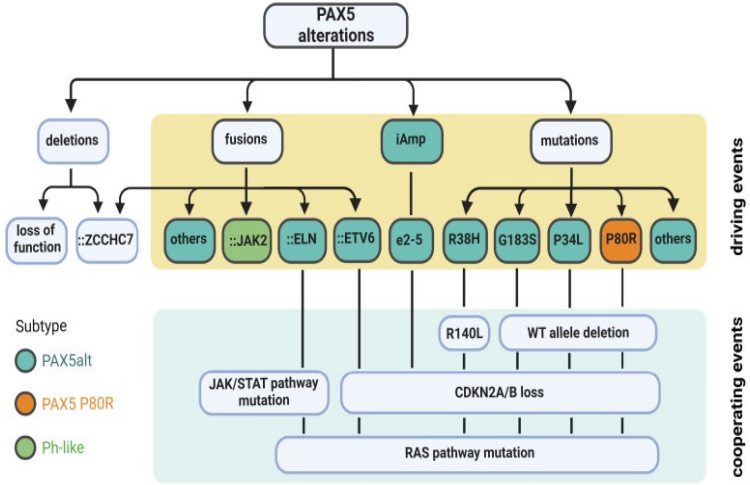
Disclosures of Nicolò Peccatori

Company name	Research support	Employee	Consultant	Stockholder	Speakers bureau	Advisory board	Other

Background

- *PAX5*-Alt ALL: a novel molecular ALL subtype
- *PAX5*-r ALL: 3-4% of pediatric B-ALL
- Intermediate outcomes for *PAX5*-Alt
- Limited evidence on the prognostic value of *PAX5* fusion genes in pediatric ALL



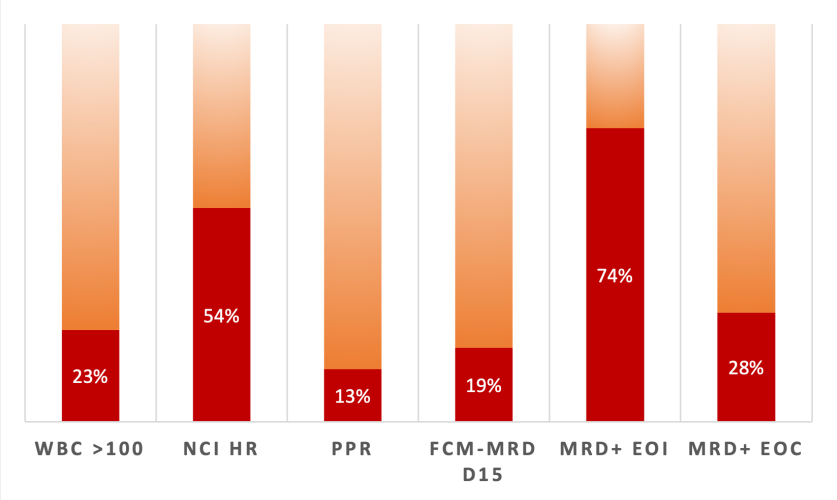
Multinational retrospective study to investigate clinical and prognostic value of PAX5-r in pediatric ALL

- 162 newly diagnosed pediatric *PAX5*-r ALL patients
- Consecutive AIEOP-BFM ALL trials (2000, 2009, 2017)
- 2002-2024 in Italy, Germany and Austria

- *PAX5* fusion genes were identified
  - Retrospectively by cytogenetics, FISH, SNP-array or targeted RNA-seq until 2018
  - Prospectively by WTS or targeted RNA-seq from 2018 onwards

Clinical and biological features of PAX5-r ALL

- Median age 3.4 years; males 66.7%
- High rates of hyperleukocytosis and NCI HR
- Tendency toward slow treatment response



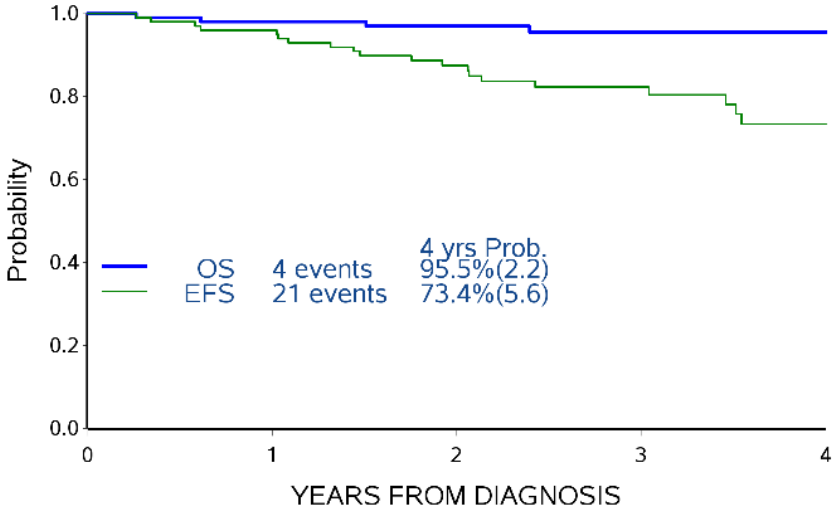
- Enrichment in IKZF1plus profile (15.7%)
- 65 different PAX5 fusion gene partners (JAK2, NOL4L, AUTS2, ETV6…)

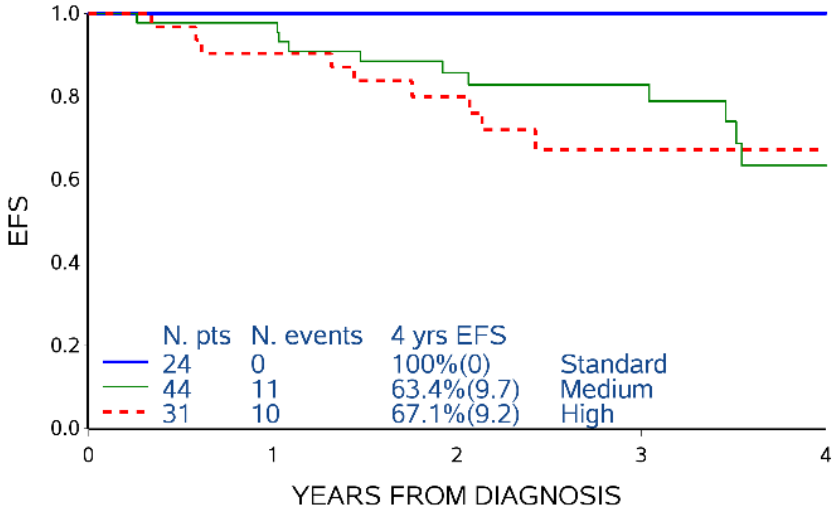
Comparative analyses in the AIEOP-BFM ALL 2017 trial

- 99 *PAX5*-r *vs* 1495 non-*PAX5*-r ALL patients (*non-ETV6::RUNX1*)
- Hyperleukocytosis, NCI HR and IKZF1^{plus} status were statistically significant higher in the *PAX5*-r ALL
- No differences in terms of MRD-based treatment response and risk stratification

Outcomes

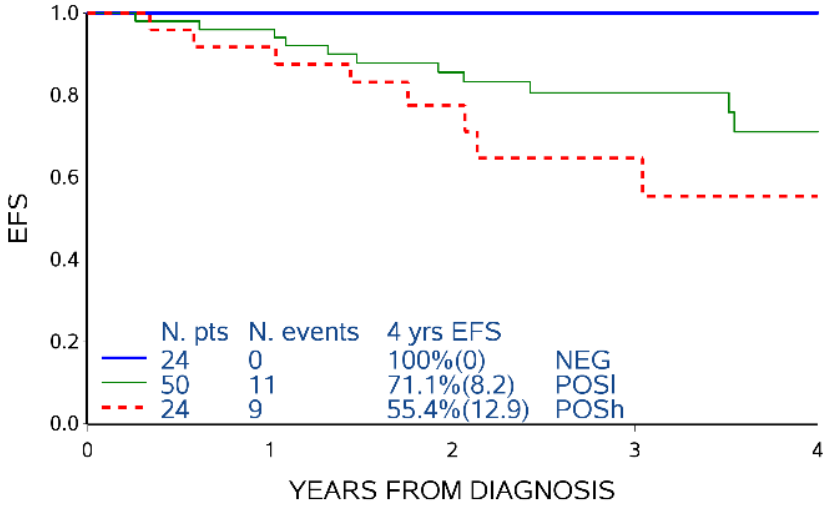
- 99 *PAX5*-r ALL pts enrolled in the AIEOP-BFM ALL 2017 trial (median FU 3.2 yrs)



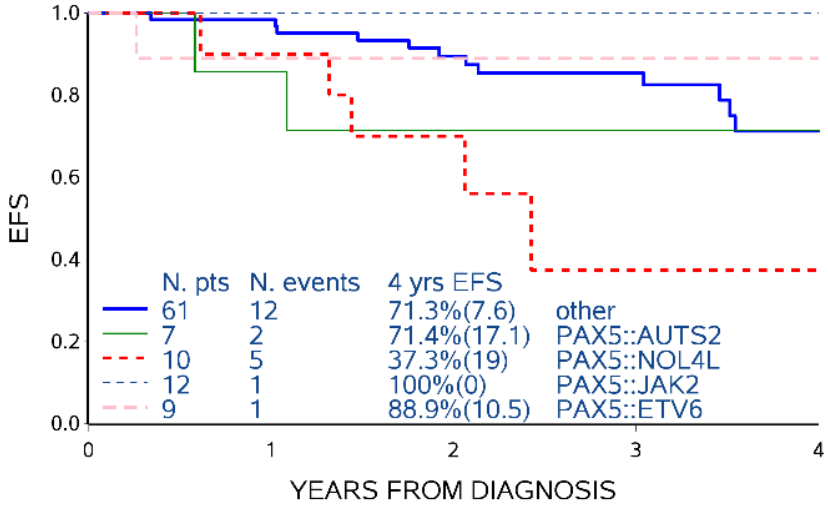


Outcomes

By EOI-MRD



By PAX5 fusion gene partner



Conclusions

Our data redefine pediatric *PAX5*-r ALL as a high-risk molecular subgroup of B-ALL, characterized by an MRD-dependent poor prognostic profile.

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FLT3 AS A NOVEL THERAPEUTIC TARGET IN PRECLINICAL MODEL OF PEDIATRIC PAX5-REARRANGED BCP-ALL

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AIRC

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