



From Sequencing to Survival: The Growing Role of Precision Medicine in Paediatric Oncology

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Abstract

The advent of comprehensive genomic sequencing has catalysed the emergence of paediatric precision medicine platforms globally. These have established the feasibility of profiling tumours at scale, enhancing our understanding of the genetic landscape of paediatric tumours and enabling identification of driver mutations and actionable targets. In turn, this has created the opportunity for development of novel precision-guided therapies (PGT). This commentary synthesizes evidence from major collaborative trials with a focus on outcome reporting, particularly in the relapsed/refractory and high-risk patient cohorts. Patients receiving PGT in these cohorts demonstrate meaningful responses and survival benefit, particularly when treatment is based on high-level clinical evidence and administered early in the disease course. However, challenges remain in addressing low uptake of PGT, likely hindered by substantial barriers in access and complex pharmaceutical regulatory constraints. Furthermore, heterogeneity in recommendation and outcome reporting hinders data harmonisation and generalisability of results. In addition to improving outcomes, comprehensive profiling can contribute to diagnostic refinement and identification of germline variant detection in a subset of patients. Emerging studies, conducted through national initiatives, signify the potential benefit of precision medicine for all patients with childhood cancer regardless of risk. A dynamic approach to address challenges and ensure cost–benefit is necessary to embed precision oncology as a standard of care for all children with cancer.

Key Points

Large national and international collaborative trials now show clinical benefit of comprehensive genomic profiling in patients with high-risk and advanced disease.

Molecularly guided therapies are most beneficial when used in the context of high-level supportive evidence and early in the disease course.

Future directions should focus on evaluating and maximising clinical benefit across all children with cancer and utilising other precision oncology non-genomics assays.

1 Introduction

Globally, cancer remains a leading cause of childhood morbidity and mortality [1]. The emergence of next-generation sequencing has resulted in the rapid evolution of our understanding of the genomic landscape of childhood cancer [2–4] and shifted the paradigm of diagnostic and treatment strategies in childhood cancer. Paediatric precision medicine platforms have been developed worldwide, allowing for the identification of molecular drivers of cancer and potential for precision-guided therapies (PGT). The evidence of benefit of these platforms in altering treatment options for children in high-risk cancer groups is growing, offering new insights into disease biology and driving discovery of novel, less toxic therapies [5]. However, significant challenges remain in accessing PGT for paediatric patients given the lack of paediatric-specific clinical trials, regulatory barriers and limited off-label availability of targeted agents [6]. In this commentary, we examine evidence from major collaborative trials that demonstrate improved clinical outcomes, particularly for those patients with high-risk disease receiving PGT on the basis of high-level clinical evidence early in their

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disease course, and advocate for comprehensive genomic sequencing in all paediatric patients with cancer.

2 Establishing Platforms

Over the last decade, precision oncology programs have been established with the aim of developing analytical pipelines for the comprehensive genomic profiling of childhood cancer. Advances in technologies have enabled the rapid analysis of tumour genomes and identification of actionable mutations in paediatric cancers. Large-scale collaborative initiatives, such as the ZERO Childhood Cancer program in Australia [2, 5], National Cancer Institute-Children's Oncology Group (NCI-COG) Pediatric MATCH (PedMATCH) [7] and Genomic Assessment Informs Novel Therapy Consortium (GAIN)/iCat2 [8] trials in the USA, SickKids Cancer Sequencing program (KiCS) in Canada and INdividualized Therapy FOr high-Risk childhood Malignancies (INFORM) [9, 10] and Molecular Profiling for Pediatric and Young Adult Cancer Treatment Stratification (MAPPYACTS) [11] studies in Europe, have cemented the role of precision medicine for children with relapsed/refractory or high-risk cancers. Collectively these studies have demonstrated successful sequencing of the vast majority of patients enrolled using a variety of next-generation sequencing techniques, including targeted panels, whole-exome sequencing (WES), whole-genome sequencing (WGS) and RNA sequencing (RNAseq) [2, 5, 7–11], and delivering results in a clinically relevant timeframe (3–6 weeks) with increasingly shorter reporting time as each platform improved its efficiency [8, 9, 12]. However, the clinical uptake of PGT recommendations remains relatively low, ranging from 10 to 33% [8, 9, 13–16]. Furthermore, most of the early studies only provided examples of responses to PGT without objective response assessments or long follow-up [14, 17–19].

3 Informing Treatment

The integration of real-time genomic curation and molecular tumour boards has improved the translation of sequencing results into therapeutic recommendations. Typically, molecular tumour boards comprise a multidisciplinary team of experts that meet weekly and review and interpret molecular profiling results to guide PGT recommendations, although each format is unique for each platform [5, 8, 10, 11]. PGT recommendations are made on the basis of the clinical relevance of genomic alterations, pre-clinical or clinical evidence of efficacy of agents combined with consideration of access to PGT and feasibility of clinical trial enrolment [5, 9, 20]. Each platform ascribes a tiering system of

recommendations that aids clinicians in prioritising potential therapies to deliver personalised medicine.

4 Clinical Benefits of Precision-Guided Treatment

Most paediatric studies have described the success of precision medicine platforms in identifying actionable alterations, but with a smaller number of patients receiving PGT and without objective outcome measurements [13–16, 18, 19]. To objectively evaluate the clinical benefits of PGT, two recent large collaborative studies have systematically reported on objective responses [8, 11], with a third study comparing progression-free survival between PGT and non-PGT [9] and a fourth study evaluating both objective responses and survival outcomes [5] (Table 1). A further study, designed to facilitate evaluation of molecular-targeted therapies in biomarker-selected cohorts, includes multiple associated treatment arms, of which some have been reported [7].

4.1 MAPPYACTS

The European-based prospective, multicentre precision medicine trial, MAPPYACTS, assessed the feasibility and clinical utility of comprehensive molecular profiling in children and adolescents with relapsed or refractory cancers [11]. WES, RNAseq and/or panel sequencing was used to identify targetable mutations and suggest salvage PGT. The study assessed the outcome of patients receiving PGT in patients with a minimum of 12 months of follow-up.

For the 787 patients consented, molecular profiling was successful in 632 samples (76%) from 624 patients (81%), with some patients undergoing second or third procedures for repeat sequencing. Potentially actionable targets were identified in 436 of 632 (69%) samples in 432 of 624 (69%) patients. Of the patients receiving a treatment recommendation, 356 had 12 months of follow-up, and 107 (30%) received 122 matched targeted therapies. Most patients (56%) received their treatment through phase I/II clinical trials. Treatment recommendations were considered “ready for routine use” in the setting of reported clinical activity, “investigational” if unestablished or limited clinical activity reported and “hypothetical” if only preclinical data existed. Most patients ($n = 97$, 80%) received an “investigational” treatment recommendation, with 44 (10%) receiving a “ready for routine use” recommendation. While the overall objective response rate (ORR) for all evaluable patients receiving PGT was 17%, the group receiving a “ready for

Table 1 Overview of large national precision medicine platforms reporting survival or response outcomes

Cohort	Sequencing assay	Successful analysis (<i>N</i> , patients)	Actionable alterations, <i>N</i> (%)	Received PGT, <i>N</i> (%)	Objective response assessment	Survival analysis	Change or refinement in diagnosis	Germline mutations
MAPPY-ACTS [11]								
Recurrent/refractory cancer Age: 6 months–18 years	WES RNAseq	624	432 (69%)	107/432 (25%)	109 cases evaluable: 18 PR (17% ORR) 27 SD (25%) DCR 41%	Not performed	20/624 (3%)	51/674 (8%)
GAIN/iCat2 [8]								
Relapsed/refractory or high-risk extracranial tumours (expected 2-year PFS ≤ 50%)* Age: ≤ 30 years	Panel +/- RNAseq	345	240 (70%)	29/240 (12%)	29 cases evaluable 5 PR (17% ORR) 2 SD (7%) OCB 24%	Not performed	17/345 (5%)	35/160 (22%)
INFORM [9]								
Relapsed, progressive or high-risk cancer Age: ≤ 21 years	WES lcWGS RNAseq Methylation	926	446 (48%)	147/446 (33%)	Not performed	Median PFS: PGT versus non-PGT (<i>p</i> = 0.97) Very high-level target 204 days versus all other 117 days (<i>p</i> = 0.011)	17/257 (7%)	39/519 (8%)
ZERO [5]								
High-risk cancer (expected cure rate < 30%) Age: ≤ 21 years	WGS RNAseq Methylation	384	256 (67%)	110/256 (43%)	70 cases measurable: 6 CR, 19 PR (36% ORR) 24 SD (34%) 97 cases evaluable for OCB: 55%	2-year PFS: PGT 26% versus non-PGT 12% (<i>p</i> = 0.049) PGT 26% versus UGT 5% (<i>p</i> = 0.003)	11/247 (4%)**	40/247 (16%)**

CR complete remission, *DCR* disease control rate, *lcWGS* low-coverage whole-genome sequencing, *NGS* next-generation sequencing, *OCB* objective clinical benefit, *ORR* objective response rate, *PD* progressive, *PFS* progression-free survival, *PR* partial response, *RNAseq* RNA sequencing, *SD* stable disease, *UGT* molecularly unguided therapy, *WES* whole-exome sequencing, *WGS* whole-genome sequencing

*Also included uncertain diagnosis

**Previously reported in a smaller cohort of 247[2]

routine use” recommendation had an ORR of 38% (5/13). The most common agents included inhibitors of WEE1 (*n* = 150), mTOR (*n* = 123), CDK4/6 (*n* = 105), MEK (*n* = 95), PARP (*n* = 64), BET (*n* = 59), EZH2 (*n* = 38), FGFR (*n* = 31) and PD1/PDL1 (*n* = 31).

4.2 GAIN

In the USA, the Genomic Assessment Informs Novel Therapy Consortium (GAIN)/iCat2 study was a multicentre observational prospective study enrolling patients aged ≤ 30 years with relapsed/refractory or high-risk extracranial

solid tumours as well as those with uncertain diagnosis after standard testing [8]. As opposed to European and Australian programs where fresh or snap-frozen tissue is preferred, this study used targeted DNA and RNA next-generation sequencing (NGS) panels on formalin-fixed paraffin-embedded (FFPE) tissue to reflect typical clinical practice in the USA. RNAseq was only performed in selected cases where it was likely to provide clinical benefit such as clinical trial eligibility.

Of the 345 patients with samples successfully sequenced, 298 (86%) had one or more alterations with at least one clinical impact across several categories; diagnostic clarification ($n = 17$, 5%), prognosis ($n = 56$, 16%), PGT recommendation ($n = 240$, 70%), PGT received with response ($n = 7$, 2%) or without response ($n = 22$, 6%). PGT uptake in those with actionable targets was the lowest of all studies ($n = 29/240$, 12%); however, of those receiving PGT with evaluable response, the ORR was 17%, and overall clinical benefit (OCB) was 24%.

4.3 INFORM

The German-based multinational INFORM registry uses WES, low-coverage WGS, DNA methylation analysis and RNAseq platform to inform actionable genetic variants for clinical trial enrolment or experimental treatment approaches [10]. PGT recommendations are made using a seven-scale target prioritisation algorithm relying on functional molecular biological relevance. The study has reported outcomes for 519 patients who have completed at least 2 years of follow-up [9, 10].

In this cohort, 147 patients received a total of 185 PGTs. Only a small number of patients received PGT recommendations with very high-level evidence, that is, clinically proven effective targeted drugs ($n = 42$, 8%). Patients receiving ALK inhibitors, BRAF inhibitors and NTRK inhibitors achieved a statistically significant improvement in progression-free survival (PFS) ($p = 0.012$) and overall survival (OS) ($p = 0.036$) compared with those harbouring *ALK*, *BRAF* or *NTRK* mutations who did not receive PGT [10]. There were no differences in PFS or OS between patients receiving PGTs across all seven evidence levels and those receiving conventional treatment or no treatment [9].

4.4 ZERO

The Australian ZERO Childhood Cancer PReCISion Medicine for Children with Cancer (PRISM) trial has recently published results highlighting the significant benefit of accessing PGT in children with high-risk paediatric cancers, defined as those patients with expected cure rate of < 30% [5]. These patients had their tumours molecularly profiled using WGS (paired tumour-germline), RNA-seq and

DNA methylation. In total, 384 patients were included in the analysis, and those who were alive at data cut-off had at least 18 months of follow-up. In total, 256 (67%) received at least one PGT recommendation, and 110 patients (43%) were treated with PGT, the highest uptake amongst paediatric precision oncology studies.

In total, 90 PGTs were evaluable for ORR, with complete remission (CR) achieved in 9%, partial remission (PR) in 21% and stable disease (SD) in 38%. In addition, 32% had progressive disease (PD). OCB, defined by complete response (CR), partial response (PR) or stable disease (SD) ≥ 24 weeks, was observed in 53 of 97 patients (55%) receiving PGT and was similar across tumour types. PGT significantly improved PFS when compared with patients who received novel targeted therapies that were not molecularly selected on the basis of the tumour profile (2-year PFS 26% versus 5.2%; $p = 0.003$) and with patients who received standard-of-care regimens (2-year PFS 26% versus 12%, $p = 0.049$). Furthermore, PGT given prior to PD resulted in significantly improved survival compared with PGT given after PD (2-year PFS 42% versus 12%; $P < 0.0001$ and 2-year OS 53% versus 29%; $P = 0.0002$). Other favourable factors for improved PGT response included PGT based on tier 1 evidence (OCB 74%) and PGT targeting fusions (OCB 75%).

4.5 NCI-COG Paediatric Match (PedMATCH)

The PedMATCH trial enrolled patients aged 1–21 years with relapsed or refractory solid tumours, lymphomas or histiocytic disorders [7]. Tumour DNA and RNA were profiled using an Oncomine AmpliSeq cancer gene panel and selected immunohistochemistry. A total of 13 treatment arms were opened. Of the first 1000 tumours screened, actionable alterations relevant to treatment arms were detected in 31.5%, with treatment arm assignment made for 28.4% of those screened. Subsequently, 13.1% of patients were enrolled in those studies. The most frequently activated alterations were found in the MAPK signalling pathway (11.2%). All treatment arms are now closed, with results being reported individually. Arm B, testing erdafitinib, a selective FGFR inhibitor, suggested possible benefit in *FGFR1*-mutant low-grade gliomas or glioneuronal tumours (PR or SD in 6/11, 54%) [21]. Arms for vemurafenib [22], olaparib [23] and ulixertinib [24] were closed for low accrual, with no OR seen, while arms for tazemetostat had 1 PR (5%) [25], and arms for selumetinib [26] and palbociclib [27] had no OR observed. Other arms are yet to be reported.

4.6 Summary

Collectively, these trials reaffirm the feasibility of comprehensive genomic profiling in relapsed/refractory or high-risk tumours, while also highlighting the high number of patients

(48–70%) with potentially targetable mutations [5, 8, 9, 11]. Uptake of PGT recommendations was variable (12–43%), potentially skewing interpretation of results [5, 8, 9, 11]. However, in all studies, except for PedMATCH, meaningful benefit in both response and/or survival outcomes was demonstrated for those receiving PGT as demonstrated by ORR ranging from 17% up to 36% across the large studies and improvements in PFS [5, 8, 9, 11]. This was particularly evident for those with strong clinical evidence to support the efficacy of the recommendation, such as those with fusions or structural variants [5], activating alterations of *ALK*, *BRAF*, and *NTRK*, [10, 11, 28] and those receiving PGT early in their disease course, before progressive disease [5]. The inferior results of some of the PedMATCH arms may be related to relapse/refractory measurable disease owing to study selection criteria and restriction to patients with disease progression. This suggests that very-high-risk patients should be considered for PGT as part of their upfront treatment instead of waiting for progression.

Only two of the large studies (ZERO and INFORM) reported survival outcomes with differences in PFS impact. ZERO reported a significant improvement in PFS between PGT and non-PGT, while INFORM described that the effect was only seen in patients receiving high-level evidence PGT. This may relate to the higher number of patients enrolled in the ZERO program at initial diagnosis (42% versus 9.6%), again suggesting that patients benefit from receiving PGT earlier in their disease course [5, 9]. The overall impact on OS is unclear, with the difference in 2-year OS in the ZERO cohort not reaching statistical significance between those receiving PGT, non-PGT, unguided molecularly targeted therapies and standard of care [5]. This may have been impacted by patients receiving multiple lines of therapy and different salvage therapies. In addition, in INFORM, patients receiving the four most frequently applied drug classes (MEK inhibitors, CDK inhibitors, mTOR inhibitors and other kinase inhibitors) did not demonstrate an improved PFS or OS, although the non-investigational nature of the registry needs to be considered in interpreting these results [10]. There were also differences in PGT recommendation approach which could have impacted treatment decisions and patient responses. INFORM's general approach was to identify therapeutic targets but refrain from making recommendations. In comparison, ZERO incorporated extensive MTB discussion with participation of the treating clinician, scientists and subject matter experts, and considered recommendations only if there was a reasonable likelihood of drug access.

Response rates in the above paediatric precision oncology trials are comparable to, and in some cases surpass, those reported in tumour-agnostic genomic profiling platforms for adult advanced cancers. The NCI-MATCH trial, enrolling over 6000 patients, identified actionable mutations

with targeted therapies available in 37.6% of patients successfully sequenced [29]. A total of 38 treatment arms were opened, enrolling 17.8% of patients screened. Overall, 7 of the 27 arms reported so far (25.9%) met the prespecified criterion for positivity, with ORR in these arms ranging from 16–50% in arms targeting *ALK* mutations, *BRAF*^{V600} mutations, *FGFR* fusions or mutations, *AKT* mutations, *PI3KCA* mutations and mismatch repair-deficient tumours [30–36]. Other treatment arms had lower response rates, though some may warrant further investigation as single agents in specific tumour types or in combination with other agents [29]. The MD Anderson Initiative for Molecular Profiling and Advanced Cancer Therapy (IMPACT) identified actionable targets in 44.3% (637/1436) of patients [37]. Patients who were treated with matched targeted therapy had higher rates of CR and PR (11% versus 5%, $p = 0.0009$), longer failure-free survival (3.4 versus 2.9 months, $p = 0.0015$) and longer OS (8.4 versus 7.3 months, $p = 0.041$) than did unmatched patients. While disease biology of adult cancers differs significantly from paediatric tumours, these studies demonstrate the feasibility and high yield of genomic profiling in identifying actionable targets as well as potential clinical benefit for adult patients with advanced cancers.

5 Future Directions

There is an emerging role for comprehensive genomic profiling in relapsed/refractory or high-risk paediatric cancer cohort. However, there remain significant challenges to generate evidence and compare results between different studies to determine whether precision medicine approaches improve patient outcomes. The direct comparison of PGT with non-PGT therapy is challenging owing to large clinical, genomic and therapeutic heterogeneity seen in this patient cohort. There is often variability between clinical studies, limiting direct comparison and data harmonisation. For example, outcome reporting is heterogeneous: In the above studies, only the MAPPYACTS and ZERO programs report responses using validated response assessment criteria such as Response Evaluation Criteria in Solid Tumors (RECIST), Response Assessment in Neuro-Oncology (RANO), PET Response Criteria in Solid Tumors (PERCIST) and International Neuroblastoma Response Criteria (INRC). Neither MAPPYACTS nor GAIN/iCat2 studies had a comparative cohort. This limits the generalisability of reported findings. Furthermore, tiering systems for recommendations are not uniform across the trials. Future trials should endeavour to standardise reporting outcomes to allow cross-series comparison and better generalisability.

Access to novel agents needs to improve. Historically, barriers to PGT access in paediatric oncology have included poor access to clinical trials, unavailability of paediatric

dosing either as monotherapies or in combination, lack of US Food and Drug Administration (FDA) approval or paediatric dosing and reluctance of clinicians and families to take up experimental therapies [5, 8, 10, 14, 18, 19]. In the ZERO and INFORM cohorts, most patients accessed drug on an off-label basis or through compassionate access programs [5, 9]. In the GAIN study, 10% of PGT therapies were not accessed owing to the drug being inaccessible [8]. In contrast, in the MAPPYACTS trial, a high proportion (56%) of patients were able to receive PGT through a clinical trial, including 72% of those through the AcSé-ESMART platform trial developed specifically for the purpose of delivering PGT to those identified through the MAPPYACTS platform [11]. This approach, while relying on buy-in from pharmaceutical companies, highlights the benefits in establishing companion basket trials with concurrent treatment arms which cater for a broad spectrum of actionable targets.

Across all programs, molecularly targeted therapies are recommended on the basis of best-available evidence at the time of case curation, relying on both clinical and pre-clinical evidence, or, when such evidence is lacking, expert consensus. A notable trend across studies is the change in recommendation tiers over time. For instance, in the GAIN/iCat2 study, the recommendation for WEE1 inhibitors in samples with *TP53* variants was downgraded from tier 2 to tier 5 [8]. Similarly, WEE1 inhibitors were the most commonly recommended agents in the MAPPYACTS study [11]. These higher-tier earlier recommendations were previously made on the basis of a phase 1 clinical trial showing modest enrichment of *TP53* variants in responders to WEE1 inhibitors [38]. However, this was not replicated in subsequent studies. As a rapidly evolving field, treatment recommendations will need to remain dynamic and responsive to emerging evidence.

The benefits of precision medicine extend beyond the identification of targetable molecular alterations. Diagnostic clarification is reported in up to 8% patients who have undergone comprehensive sequencing [2, 8, 11, 39]. DNA methylation, included in the profiling suite of some major platforms, has reshaped the classification of both central nervous system tumours and sarcomas, improving diagnostic accuracy and therefore impacting treatment [40, 41]. Germline variants were also identified in as many as 22% of patients who underwent germline analysis across the studies [2, 8, 9, 11, 39, 42].

Evidence is now starting to emerge as to the benefit of precision medicine for all children diagnosed with cancer, regardless of risk, through national initiatives such as the Genomic Medicine Service within the National Health Service (NHS) in England [39], Genomic Medicine Sweden

(GMS) Childhood Cancer project [42] and Zero Childhood Cancer Program in Australia (NCT05504772). Both the English and Swedish platforms have demonstrated reproduction of standard-of-care clinical testing while also identifying the additional benefits of a comprehensive platform. For example, the Swedish GMS Childhood Cancer project, using a tumour and germline WGS and RNA-seq pipeline for 117 patients, reported additional subclassification or detection of prognostic markers in 50% (59/118) of solid tumour samples when compared with standard clinical testing and identification of actionable molecular targets in 26% (31/118) [42]. From England, Hodder et al. reported 282 samples sequenced from 281 patients, primarily enrolled at diagnosis with solid and haematological malignancies [39]. In this cohort, additional disease-relevant findings were seen in 29% of cases (83/282), including 20 cases (7%) in whom a unique WGS finding changed clinical care.

The scope of precision oncology is expanding rapidly beyond genomic profiling alone. Transcriptional signatures and biomarker-driven clinical trials are being used to identify potential response to immunotherapies, which have thus far proved disappointing in most paediatric cancers [43, 44]. For example, the paediatric Optimal Precision Therapies to CustoMISE Care (OPTIMISE) basket trial in Australia (NCT06208657) will be using transcriptomic immune signatures as biomarkers in the upcoming immunotherapy arm (nivolumab and relatlimab combination) [43, 45, 46]. Large platforms are accelerating functional studies, such as proteomics and drug screening to facilitate identification of novel biomarkers and therapeutic targets [11, 47–50]. Liquid biopsy programs provide enhanced opportunities for risk stratification and disease monitoring [11, 51]. Furthermore, artificial intelligence offers the potential to improve diagnostic accuracy, risk stratification and treatment response prediction, using complex algorithms such as machine learning and deep learning techniques [52, 53].

Consideration must be made to the cost of delivering such platforms. As suggested by Church et al., targeted sequencing panels may be more attractive in resource-limited settings owing to lower costs, and less labour-intensive computational and curation requirements [8]. We propose that increased investment in sequencing technologies and higher volume of patients undergoing molecular profiling can lower the cost per sample by leveraging economies of scale and improving workflow efficiency. Cost–benefit and health economics analyses embedded within future trials will help inform clinical and ethical frameworks for the implementation of wide-scale comprehensive genomic sequencing in paediatric oncology.

6 Conclusions

The growing body of evidence from large-scale paediatric precision oncology initiatives underscores the transformative potential of comprehensive genomic profiling in paediatric cancer care. These platforms have successfully sequenced thousands of tumours, resulting in identification of actionable targets, informed personalised treatment strategies and meaningful objective responses in high-risk cohorts. In addition to improving outcomes, in a subset of patients, comprehensive profiling has added diagnostic clarification and identified germline mutations and cancer predisposition syndromes, further enhancing its clinical value.

Future studies should focus on consolidating and harmonising data outcome reporting to further strengthen the generalisability of results. Studies should include integrated cost–benefit analysis to establish feasibility and support implementation for all paediatric patients with cancer, in addition to high-risk cohorts. Crucially, expanded clinical trial infrastructure and streamlined regulatory pathways are essential to overcoming current barriers to accessing novel therapies. The scope of precision oncology is also expanding to include proteomics, liquid biopsy, tumour microenvironment evaluation and functional studies for biomarker identification, disease monitoring and treatment. As the field continues to evolve, a dynamic and adaptive approach will be necessary to embed precision oncology as a standard of care for all children with cancer.

Declarations

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