Efficacy and Safety of Erdafitinib in Pediatric Patients with Advanced Solid Tumors and *FGFR* Alterations in the Phase 2 RAGNAR Trial

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Presented by: O Witt at the 2024 ASCO Annual Meeting; May 31- June 04, 2024; Chicago, IL, USA

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CONCLUSIONS

- In a small pediatric cohort (N=11) consisting primarily of refractory HGG and LGG with *FGFR* alteration, erdafitinib was associated with clinically meaningful disease control with acceptable safety
  - Investigator-assessed ORR was 9.1%, DCR 72.7%, and mPFS 29.5 months
- These findings support the continued research of *FGFR* inhibitors in pediatric patients with advanced solid tumors who harbor susceptible *FGFR* alterations and have exhausted alternative treatment options

DCR, disease control rate; *FGFR*, fibroblast growth factor receptor; HGG, high-grade glioma; LGG, low-grade glioma; mPFS, median progression-free survival; ORR, objective response rate

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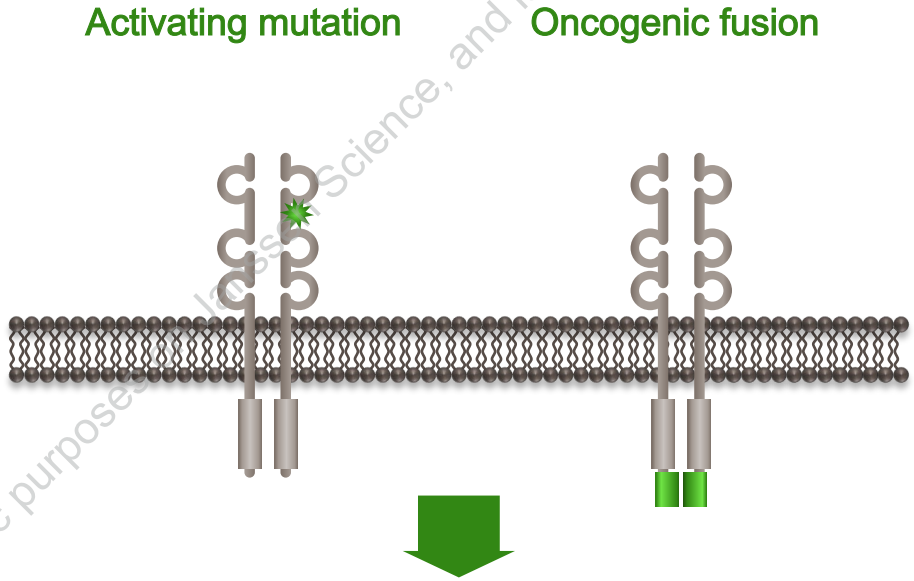
INTRODUCTION

- *FGFR* gene alterations representing potentially targetable genomic variants[1,2] occur in **~3%** of pediatric solid tumors[3]
- Erdafitinib is an oral selective pan-*FGFR* TKI approved by the FDA for the treatment of adults with locally advanced or metastatic urothelial carcinoma with susceptible *FGFR3* genetic alterations who have progressed on or after ≥1 line of prior systemic therapy
- Primary analysis of the RAGNAR (NCT04083976) study showed an **ORR of 30%** (95% CI, 24-36) and **mDOR of 6.9 months** (95% CI, 4.4-7.1) in solid tumors with predefined *FGFR* mutations or fusions[4]
- **Objective:** We present the efficacy and safety outcomes from the Pediatric Cohort of the RAGNAR study



Oncogenesis

Cancer cell proliferation, metastases, and angiogenesis

1. Parsons DW, et al. *J Clin Oncol*. 2019;37:15_suppl; abstr 10011. 2. Gröbner SN, et al. *Nature*. 2018;555:4733-4740. 3. Lazo De La Vega L, et al. *JCO Precis Oncol* 2022;6:e2200390. 4. Pant S, et al. *Lancet Oncol*. 2023;24:925-935.
CI, confidence interval; FDA, Food and Drug Administration; *FGFR*, fibroblast growth factor receptor; mDOR, median duration of response; ORR, objective response rate; TKI, tyrosine kinase inhibitor

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METHODS

Study Participants (Pediatric Cohort)

- RAGNAR (NCT04083976): a tumor-agnostic, phase 2, single arm, open-label study of the efficacy and safety of erdafitinib in patients with advanced solid tumors and eligible *FGFR* alterations
- RAGNAR included a dedicated pediatric cohort
  - Planned enrollment:
    - 20 previously treated patients
    - Additional 6 newly diagnosed patients (with no SoC treatment options)

Key Inclusion Criteria

- Age ≥6 to <18 years: histologic evidence of unresectable, locally advanced, or metastatic solid tumor
- *FGFR* mutations, gene fusions, or internal tandem duplications
- Measurable disease
- Disease progression requiring treatment change before study screening or newly-diagnosed disease without acceptable standard therapies

Key Exclusion Criteria

- Prior chemotherapy, targeted therapy, or investigational anticancer agent within 15 days or <5 half-lives before first erdafitinib dose, up to 30 days prior
- Presence of *FGFR* gatekeeper and resistance mutations
- Hematologic malignancy (i.e., myeloid and lymphoid neoplasms)
- Active malignancies other than for disease requiring therapy
- Patients with previous FGFR inhibitor treatment

FGFR fibroblast growth factor receptor; SoC, standard of care

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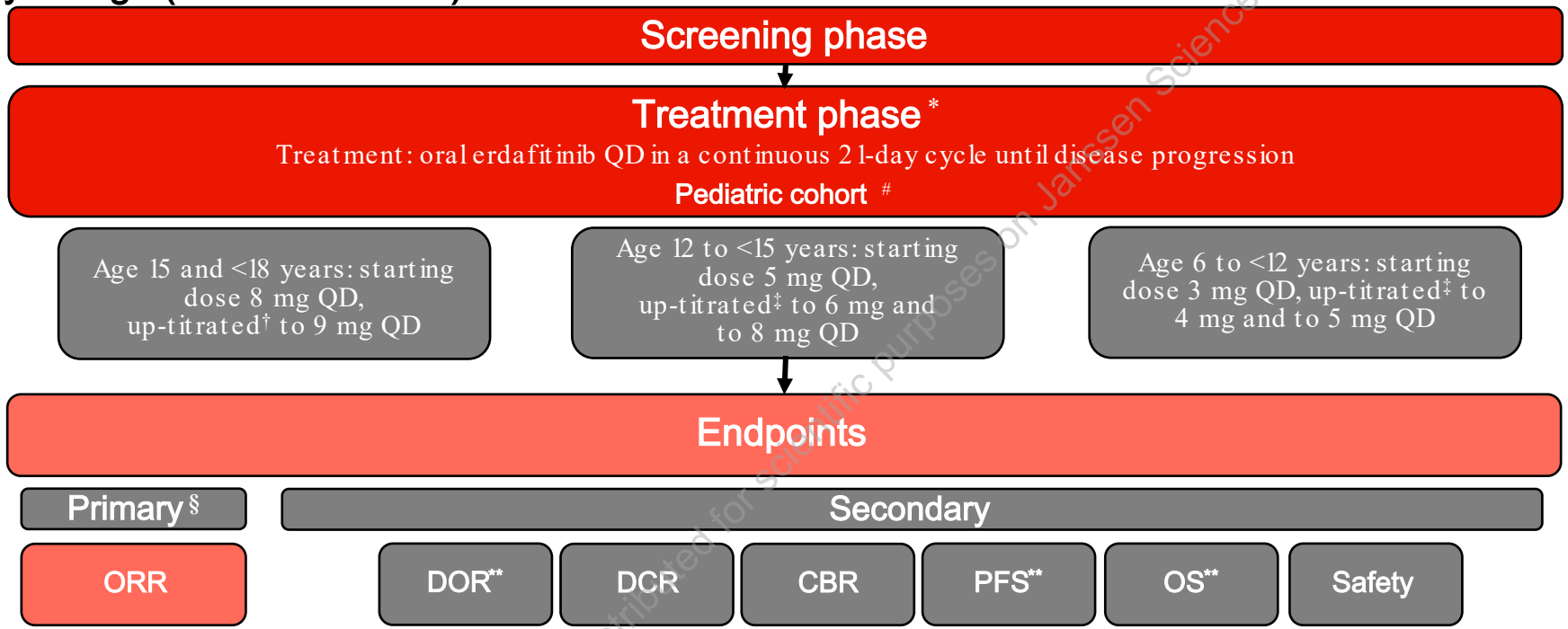
METHODS

Study Design (Pediatric Cohort)



*Treatment was continued until progressive disease, intolerable toxicity, consent withdrawal, or investigator decision to stop treatment. #Patients with *FGFR* mutations (exclusive of valine gatekeeper and resistance alterations), any *FGFR* gene fusion, or *FGFR* internal tandem duplication were eligible for enrollment in the Pediatric Cohort. †Based on cycle 1 day 14 serum phosphate concentrations. ‡Based on cycle 1 day 14 and cycle 2 day 7 serum phosphate concentrations §Evaluated per RECIST (v 1.1) or RANO and calculated with 95% 2-sided exact CIs. **Summarized using Kaplan–Meier methods
CBR: the proportion of patients with CR, PR, or durable SD of at least 4 months; DCR: the proportion of patients with CR, PR, or SD
CBR, clinical benefit rate; CI, confidence interval; CR, complete response; DCR, disease control rate; DOR, duration of response; *FGFR*, fibroblast growth factor receptor; ORR, objective response rate; OS, overall survival; PFS, progression-free survival; PR, partial response; QD, once daily; RANO, Response Assessment in Neuro-Oncology; RECIST, Response Evaluation Criteria in Solid Tumors; SD, stable disease

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RESULTS

Baseline Characteristics

- 11 pediatric patients received erdafitinib
  - LGG: 6 (54.5%)
  - HGG: 3 (27.3%)
  - STS and temporal neurocytoma: 1 (9.1%) each
- Most patients were female (7/11 [63.6%])
- Median age was 13 (range, 6-16) years
- 1/11 (9.1%) patient was newly diagnosed, and 10/11 (90.9%) patients received ≥1 previous line of systemic therapy
  - 8 (72.7%) had prior cancer-related surgery
  - 6 (54.5%) had prior radiotherapy

Treated patients	LGG n=6	HGG n=3	Other n=2	Total N=11
Age, median (range), years	10.5 (6-16)	13.0 (13-15)	15.0 (15-15)	13.0 (6-16)
Sex, female, n (%)	4 (66.7)	1 (33.3)	2 (100)	7 (63.6)
Race, n (%)				
Asian	1 (16.7)	1 (33.3)	0	2 (18.2)
Black or African American	0	1 (33.3)	1 (50.0)	2 (18.2)
White	4 (66.7)	0	1 (50.0)	5 (45.5)
Not Reported	1 (16.7)	1 (33.3)	0	2 (18.2)
Number of prior lines of anti -cancer therapies, n (%)				
1	4 (66.7)	1 (33.3)	0	5 (45.5)
2	0	2 (66.7)	0	2 (18.2)
≥3	1 (16.7)	0	2 (100)	3 (27.3)
Median (range)	1 (0-3)	2 (1-2)	4 (3-5)	1 (0-5)
Response to last line of prior systemic therapy, n (%)				
CR	2 (33.3)	0	0	2 (18.2)
PR	0	1 (33.3)	0	1 (9.1)
SD	3 (50)	1 (33.3)	1 (50)	5 (45.5)
PD	0	1 (33.3)	1 (50)	2 (18.2)

CR, complete response; HGG, high-grade glioma; LGG, low-grade glioma; ORR, objective response rate; PD, progressive disease; PR, partial response; SD, stable disease; STS, soft-tissue sarcoma

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RESULTS

FGFR Alterations

- Gene alterations observed:
  - *FGFR1* in 7 (63.6%) patients
  - *FGFR3* in 3 (27.3%) patients
  - *FGFR2* in 1 (9.1%) patient
- *FGFR* alteration types:
  - Fusions in 6 (54.5%) patients
  - Mutations in 4 (36.4%) patients
  - Tandem duplication in 1 (9.1%) patient
- Among patients with HGG/LGG tumors, *FGFR1* alterations (77.8%) and gene fusions (55.6%) were most commonly observed

	Evaluable Patients (N=11) n (%)	Altered *FGFR* Gene			*FGFR* Alteration Type		
		FGFR1	*FGFR2*	*FGFR3*	Fusion	Mutation	Duplication
Total	**11 (100)**	**7 (63.6)**	**1 (9.1)**	**3 (27.3)**	**6 (54.5)**	**4 (36.4)**	**1 (9.1)**
LGG	6 (54.5)	5 (83.3)	1 (16.7)	0	3 (50.0)	2 (33.3)	1 (16.7)
HGG	3 (27.3)	2 (66.7)	0	1 (33.3)	2 (66.7)	1 (33.3)	0
Other*	2 (18.2)	0	0	2 (100)	1 (50.0)	1 (50.0)	0

*STS and temporal neurocytoma (1 each)

FGFR fibroblast growth factor receptor; HGG, high-grade glioma; LGG, low-grade glioma; STS, soft-tissue sarcoma

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RESULTS

Tumor Response by Histology (Per Investigator)

	N	ORR n (%) [95%CI]	DCR n (%) [95%CI]	Median DOR [95% CI], months	Median PFS, months	Median OS, months
Total	11	1 (9.1) [0.2-41.3]	8 (72.7) [39.0-94.0]	19.75 [NE-NE]	29.47	NE
LGG	6	0 [NE-NE]	6 (100) [54.1-100]		NE	NE
HGG	3	1 (33.3) [0.8-90.6]	2 (66.7) [9.4-99.2]	19.75 [NE-NE]	29.47	NE
STS	1	0 [NE-NE]	0 [NE-NE]		0.53	1.02
Temporal neurocytoma	1	0 [NE-NE]	0 [NE-NE]		1.41	NE

At data cut -off (December 4, 2023) with a median efficacy follow -up of 9.7 months,

- The ORR (95% CI) was 9.1% (0.2-41.3)
  - 1 patient with HGG achieved PR lasting 19.75 months
- Durable SD ≥4 months was observed in all 6 patients with LGG and in 1/3 patient with HGG
- 9/11 (81.8%) of patients were censured for OS, and 2/11 (18.2%) died

Durable SD: defined as duration of at least 4 months

CI, confidence interval; DCR, disease control rate; DOR, duration of response; HGG, high-grade glioma; LGG, low-grade glioma; NE, not evaluable; ORR, objective response rate; OS, overall survival; PR, partial response; PFS, progression-free survival; SD, stable disease; STS, soft tissue sarcoma

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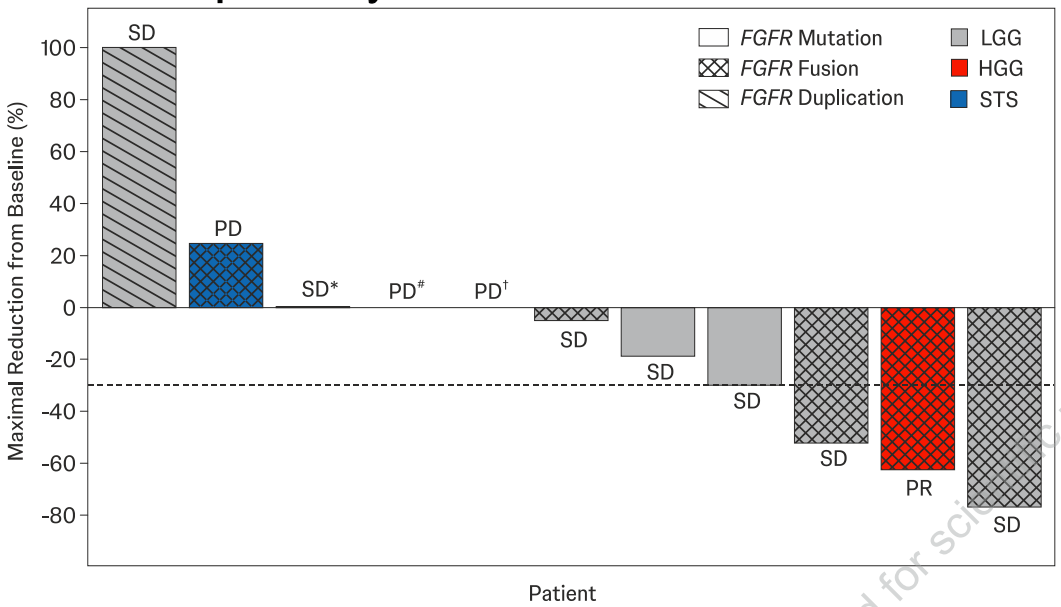
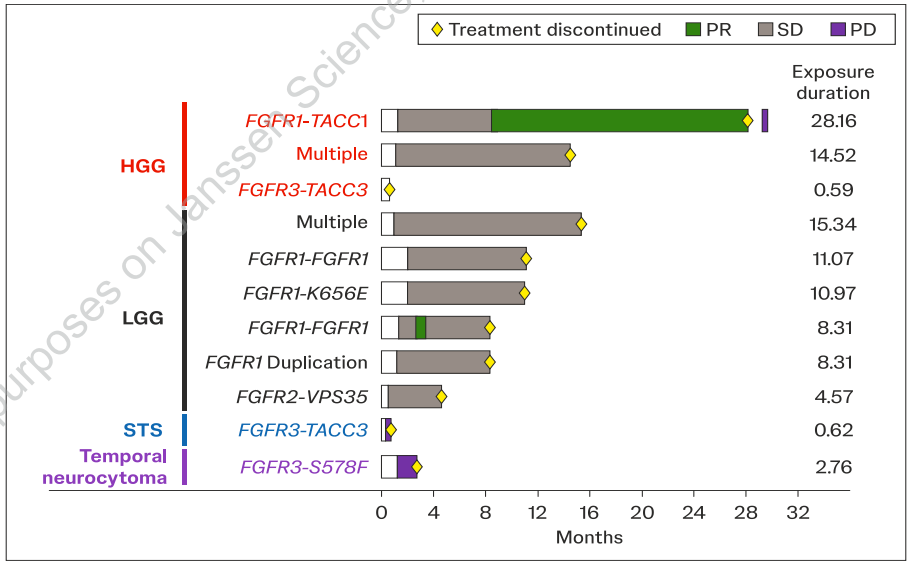
RESULTS

Tumor Response by FGFR Alterations





- Among 11 evaluable patients, tumor response or durable SD was observed in both patients with *FGFR* mutations and fusions
  - 1 patient with PR had a HGG and *FGFR1-TACC1* fusion
  - Of 7 patients with durable SD ≥4 months, 3 had *FGFR1* mutations, 2 had *FGFR1* fusions, 1 had *FGFR2* fusion, and 1 had *FGFR1* duplication

*Patient with HGG and *FGFR* mutation; # Patient with HGG and *FGFR* fusion; †Patient with temporal neurocytoma and *FGFR* mutation
FGFR fibroblast growth factor receptor; HGG, high-grade glioma; LGG, low-grade glioma; ORR, objective response rate; PD, progressive disease; PR, partial response; SD, stable disease; STS, soft tissue sarcoma

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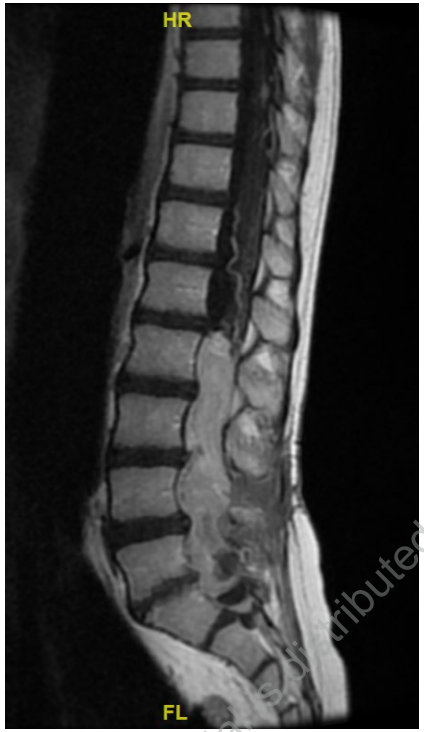
RESULTS

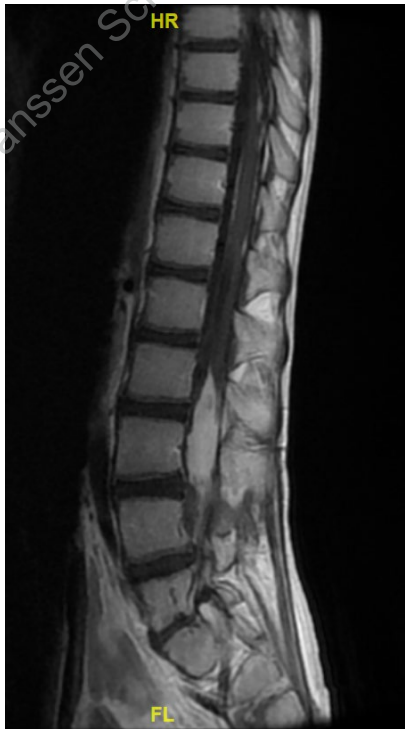
Pediatric Case Study: PR of 19.75 Months

13-year-old patient with anaplastic pilocytic astrocytoma, *FGFR1-TACC1 fusion*

MRI Baseline (Aug 9, 2020)



MRI 6 cycles (Feb 17, 2021)



FGFR, fibroblast growth factor receptor; MRI, magnetic resonance imaging; PR, partial response

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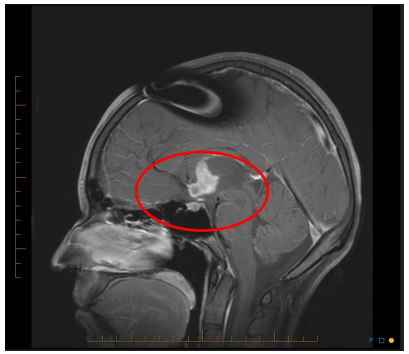
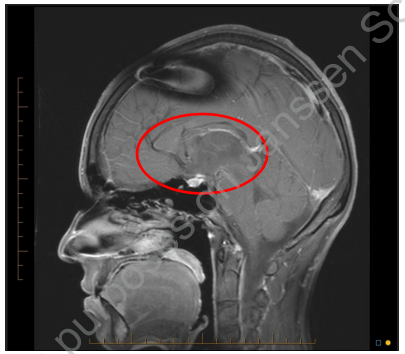
Adult Case Study: PR→CR of 29.1 Months

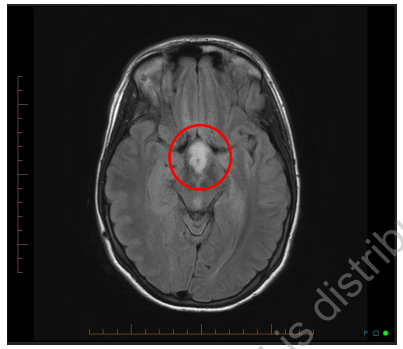
26-year-old patient with dysembryoplastic neuroepithelial tumor and *FGFR1 K656E* mutation

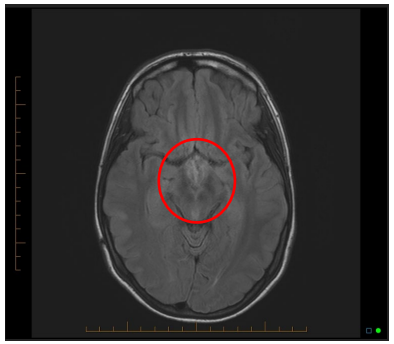


MRI (Aug 5, 2020)



MRI (Dec 31, 2020)





This patient was enrolled in RAGNAR but was not part of the Pediatric Cohort
CR, complete response; *FGFR* fibroblast growth factor receptor; MRI, magnetic resonance imaging; PR, partial response

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RESULTS

Safety

- All erdafitinib-treated patients had at least 1 TEAE
- Adverse events unique to the pediatric population were observed:
  - Events of growth disorder were reported by 4/11 (36.4%) patients
- 4 patients with limb fracture
- 1 patient additionally had Gr 3 epiphysiolysis
- No central serous retinopathy events occurred
- 72.7% (8/11) of patients had Gr ≥3 TEAEs; 5/11 (45.5%) were considered treatment-related
- Most TEAEs were managed with dose modification and symptomatic or conservative management
- 72.7% (8/11) of patients had serious TEAEs
  - 4 (36.4%) were considered treatment-related (tibia fracture, dehydration, epiphysiolysis, and neuropathy peripheral)
- No treatment-related deaths were reported

TEAE by preferred term in ≥25% of patients, n (%)	N=11	
	Any grade	Gr ≥3
Diarrhea	7 (63.6)	0
Hyperphosphatemia	7 (63.6)	1 (9.1)
Pain in extremity	5 (45.5)	0
ALT increased	4 (36.4)	0
Nausea	4 (36.4)	0

ALT, Alanine aminotransferase; Gr, grade; TEAE, treatment-emergent adverse event

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CONCLUSIONS

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  - Investigator-assessed ORR was 9.1%, DCR 72.7%, and mPFS 29.5 months
- These findings support the continued research of *FGFR* inhibitors in pediatric patients with advanced solid tumors who harbor susceptible *FGFR* alterations and have exhausted alternative treatment options

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REFERENCES:

1. Parsons DW, et al. *J Clin Oncol*. 2019;37:15_suppl; abstr 10011.
2. Gröbner SN, et al. *Nature*. 2018;555:4733-4740.
3. Lazo De La Vega L, et al. *JCO Precis Oncol*. 2022;6:e2200390.
4. Pant S, et al. *Lancet Oncol*. 2023;24:925-935.

DISCLOSURES:

Olaf Witt has received honoraria from Roche Pharma AG; consultant/advisory role with AstraZeneca, Bristol-Myers Squibb, Novartis; research funding from Janssen Research & Development, PreComb Therapeutics, Bristol-Myers Squibb/Ono Pharmaceutical.

ACKNOWLEDGMENTS:

The authors would like to thank the patients and their families for their participation in this study.

Erdafitinib (JNJ-42756493) was discovered in collaboration with Astex Pharmaceuticals.

Vaibhav Deshpande, PhD (SIRO Clinpharm Pvt. Ltd., India) provided writing assistance and Jennifer Han, MS (Janssen Global Services, LLC) provided additional editorial support.

Funding: Janssen Research & Development, LLC.

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