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Real-world experience with IDH inhibitors in pediatric patients with *IDH1*- and *IDH2*-mutated acute myeloid leukemia and myelodysplastic syndrome

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Running head: IDH inhibitors in pediatric AML and MDS

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Manuscript text:

To the editor:

IDH1 and IDH2 mutations are rare in pediatric patients, reported in 2-4% of children and adolescents with acute myeloid leukemia (AML)[1-6] and myelodysplastic syndrome (MDS).[7] Outcomes of IDH-mutated pediatric AML are relatively favorable compared to adults; however, when IDH mutations occur without favorable *NPM1* mutations, inferior outcomes have been reported in retrospective analyses.[5] The IDH inhibitors (IDHi) ivosidenib (IDH1i) and enasidenib (IDH2i) are oral inhibitors of mutant IDH protein. Based on the results of previous clinical trials, both are approved for use in adult patients with relapsed/refractory (R/R) IDH-mutated AML,[8,9] and ivosidenib monotherapy is approved for R/R-MDS.[10]

The active site mutations are the same in adult and pediatric IDH-mutated AML, supporting the biological rationale for IDHi use in pediatric patients; however, their clinical utility has not been established, largely due to the rarity of the mutations. Clinical trials of enasidenib for pediatric patients with multiply R/R-AML (NCT04203316, NCT02813135) have faced significant accrual challenges and no trials exist for ivosidenib. With the availability of IDHi via compassionate access or off-label commercial prescribing, the reportable patient experience may inform the therapeutic integration of these agents and the design of future clinical trials. In this report of real-world data, we provide the first description of pediatric patients who received ivosidenib or enasidenib for IDH-mutated AML or MDS. The institutional review board at each site approved submission of de-identified patient data to a central database. Data use agreements were executed with the coordinating center (Ann & Robert H. Lurie Children's Hospital of Chicago).

We report on nine children with IDH-mutated AML/MDS treated with IDHi in the United States and France from 2019 – 2024. Patient and disease characteristics are described for those who received ivosidenib therapy in **Table 1**, and enasidenib therapy in **Table 2**. Four patients were treated with

ivosidenib (age at IDHi use; median [range]: 12.1 [7.5 – 13.8] years), and five with enasidenib (8.3 [1.6 – 18.9] years). Initial diagnoses include *de novo* AML (n=7, 77.8%) and MDS (n=2, 22.2%). At the time of IDHi use, treatment status included relapse following allogeneic hematopoietic stem cell transplant (HCT) (n=6, 66.7%), including two patients with secondary AML after HCT for MDS, primary refractory acute megakaryoblastic leukemia (n=1, 11.1%), and relapse after first complete remission (CR1) with chemotherapy (n=2, 22.2%). No patients had extramedullary disease when IDHi was initiated, though one patient developed soft tissue chloromas during therapy. All IDH mutations were active site mutations: *IDH1*^{R132} (n=4, 100%), *IDH2*^{R140} (n=5, 100%). Cytomolecular findings including karyotype, additional somatic mutations, and IDH variant allele frequency are reported (**Table 1, Table 2, Figure 1**). Recurrent somatic mutations included *FLT3*-ITD (n=22.2%), *TP53* (n=2, 22.2%), *RUNX1* (n=2, 22.2%), and *WT1* (n=22.2%) (**Figure 1**).

Access to IDHi was obtained by commercial prescription (n=5, 55.5%) or compassionate use (n=4, 44.4%). One child (9 years of age) was treated with ivosidenib at adult dosing of 500 mg daily, two children each of 12 years of age at 250 mg daily and a 7-year-old at 150 mg daily. For enasidenib, two 8-year-old children received 50 mg daily with weight-based dosing (2mg/kg/day); the 3 others were treated at adult dosing of 100 mg daily, including the youngest child who was less than 2 years of age. Three patients received IDHi monotherapy: (1) enasidenib after several prior salvage attempts, (2) ivosidenib for six weeks then with venetoclax and (3) ivosidenib for one month then with hydroxyurea. The remainder received IDHi with a variety of chemotherapeutic agents (e.g., venetoclax, hypomethylating agents (HMA), myelosuppressive chemotherapy of varying intensity or antigen-directed therapies; **Table 1, Table 2**).

One patient developed IDH-differentiation syndrome (IDH-DS) on cycle 2, day 25 of ivosidenib monotherapy. IDH1i was discontinued and the patient received supportive care (corticosteroids, diuresis,

and respiratory support without intubation) before ivosidenib was restarted after 11 days. Ivosidenib was held a second time due to recurrent symptoms but restarted with venetoclax without interruption for two subsequent cycles. Nearly all patients experienced hematopoietic toxicity (**Table 1, Table 2**) including grade 3 anemia, grade 4 neutropenia, thrombocytopenia; one had grade 3 nausea; no patient had indirect hyperbilirubinemia.

Five patients (n=5/9, 55.5%; ivosidenib (n=2/4, 50%); enasidenib (n=3/5, 60%)) experienced CR with incomplete count recovery (CRi). For the three enasidenib-treated patients with CRi, MRD was negative by flow cytometry and *IDH2* mutation detection by next-generation sequencing (NGS); two ivosidenib-treated patients had flow MRD-positive CRi, one with *IDH1* mutation detectable by NGS, the other was unknown. One patient who received ivosidenib had a partial response (PR) and the other three patients were non-responders (**Table 1, 2; Figure 1**). The median time to best response was 79.5 days (28 – 108 days). No patient had a clinical response to IDHi monotherapy. Four patients (44.4%) underwent HCT following IDHi and two received post-HCT enasidenib maintenance therapy. One patient started IDH2i at 1.5 months after HCT but developed cytomegalovirus infection and enasidenib was discontinued after 36 days; this patient experienced non-relapse mortality one year post-HCT. A second patient started enasidenib 4.5 months after HCT and continued IDH2i for 24 months. Post-HCT bone marrow evaluations demonstrated sustained flow MRD-negative CR and serial surveillance of the peripheral blood by IDH2 NGS remained negative for the duration of planned maintenance therapy. Two patients (22.2%) were alive with median duration of response 23.5 months [15 – 40.8 months] at time of data collection.

This is the first case series to describe treatment with IDHi in pediatric patients with multiply R/R IDH-mutated AML/MDS. Prior to and with IDHi, most patients received combinations of chemotherapy of varying intensity, including HMA or venetoclax, like strategies utilized in adult studies of R/R myeloid malignancy.[11,12] Three patients who received IDHi monotherapy failed to respond in contrast to overall

response rates of approximately 40% in adult patients with R/R-AML receiving monotherapy.[8,9] Our cohort's best response rate (CRi) was 55.6% with combination approaches of varying intensity; time to response is within range of monotherapy and combination therapy in adults.[8,9,12] Given the heterogeneity of combination therapies, the specific contribution of IDHi is difficult to determine. However, all patients who received venetoclax with or immediately prior to IDHi experienced CRi, consistent with preclinical evidence of sensitivity of IDH-mutated AML to BCL2 inhibition and favorable results of combination clinical trials in adult patients,[11,12] particularly in triplet regimens with HMA.[11] Two patients treated with enasidenib who achieved a flow and NGS MRD-negative CR remained alive after HCT indicating a durable remission; the third such patient experienced non-relapse mortality precluding determination of long-term response.

Treatment with IDHi was tolerable; IDHi was discontinued due to progressive disease or transition to alternative therapy rather than toxicity. All patients had hematologic toxicities as expected given disease state and extent of prior therapies. The frequency of IDH-DS (11.1%) compares to reported rates of this IDHi treatment-related adverse event.[13] Importantly, IDH-DS was clinically recognized and recommended management was implemented.[13] In one patient post-HCT maintenance was tolerable and continued for the planned duration of two years with ongoing remission maintenance. Another patient discontinued post-HCT IDHi due to infectious toxicity likely unrelated to IDHi. Post-HCT maintenance with IDHi has thus far been safe and well-tolerated in early phase studies in adult patients.[14,15]

We previously showed that pediatric patients with IDH-mutated AML without *NPM1* mutations experienced inferior outcomes with conventional chemotherapy.[5] Consistent with this, in our R/R cohort there was a paucity of *NPM1* mutations. One patient with *IDH2* and *NPM1* also had *FLT3*-ITD at diagnosis, previously shown to abrogate the favorable impact of *NPM1* on outcomes.[5] For this patient, *FLT3*-ITD was undetectable at relapse though *NPM1* remained; treatment with enasidenib and venetoclax

resulted in MRD-negative CR consolidated by HCT. In adult trials of IDHi, fewer patients with *WT1* mutations responded to ivosidenib monotherapy,[8] and we observed non-response in our two patients; however, this finding requires validation in larger cohorts across age.

Overall, treatment with IDHi was tolerable in pediatric patients with toxicity congruent with adult trials. The response rate in our patient cohort with combination approaches, particularly with venetoclax, is encouraging given the advanced disease states represented. Based on this response rate and ability to consolidate response with HCT in several patients, combination therapies with IDHi represent attractive approaches for prospective clinical investigation. If the rarity of this genetic subset of pediatric AML/MDS precludes clinical trial development, our findings suggest that IDHi are reasonable to consider in combination with venetoclax, HMA, and/or chemotherapy in the R/R setting.

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Table 1: Ivosidenib therapy including patient and disease characteristics with treatment details and outcomes

Table 2: Enasidenib therapy including patient and disease characteristics with treatment details and outcomes

Table 1: Ivosidenib therapy including patient and disease characteristics with treatment details and outcomes

ID	Age (years) at time of IDHi use / Sex	At diagnosis			At time of IDH inhibitor use					Toxicity T=thrombo-cytopenia A=anemia N=neutropenia	Best response Flow % / IDH VAF	HCT	Alive
	Race/Ethnicity	Diagnosis, CNS status	*IDH1* VAF	*Karyotype* *Fusions, somatic mutations*	Disease state, CNS status	*IDH1* VAF	*Karyotype* *Fusions, somatic mutations*	Treatment before IDHi *with IDHi*	IDHi dose (mg) IDHi duration / reason for discontinuation		Time to best response	Post-HCT IDHi	
1	7.5/M	*de novo* AML, CNS1	42%	46,XY, t(8;21)(q22;q22)[1] 45,idem,-Y[17] 46,XY[2]	Post-HCT (in CR1) relapse, CNS1	27%	Complex[1]	Chemotherapy[2]	100 x 1 month, 150 final dose (interrupted for ICU admissions for COVID19 infection)	Gr. 3: Nausea, A	PR 7.4% / 43%	no	no
	White, non-Hispanic			*RUNX1*::*RUNX1T1* *KIT*^{N822K}, *BRCA1*^{V920I}			*RUNX1*::*RUNX1T1* *CEBPA*, *EZH2*^{E745K},*KIT*^{N822K}, *ASXL*^{W898}	*HMA + GO*	5 months / PD	Gr. 4: T, N	Day 34	n/a	
2	11.8/M	MDS, CNS1	mutation status unknown	47, XY +8	Secondary AML after HCT for MDS, CNS1	18.7%	47, XY +8	HMA + chemotherapy[3] eprenetapopt	250 x 1 week, 500 final dose	Gr. 3: A	CRi, MRD^{pos} 1.1% / unknown	yes	no
	White, non-Hispanic			*TP53, JAK2*^{V617F}			*TP53*^{G743A}, *TET2*^{T5162G}, *CS3R*^{T2087C}	*HMA + eprenetapopt + tagroxafusp* *HMA + CD74-ADC*	3.5 months / PD	Gr. 4: T, N	Day 108	no	
3	12.5/F	Hypoplastic MDS, CNS1	mutation status unknown	Unknown	Secondary AML after 2nd HCT for MDS, CNS1	VAF unknown	46,XX[14] 46,XY[6]	Venetoclax	250 (interrupted for IDH-DS)	IDH-DS Gr. 3: A Gr. 4: T, N	CRi, MRD^{pos} 2.9% / detectable	no	no
	Asian			Unknown			*KMT2A*-PTD, *RUNX1*	*Monotherapy x 6 weeks followed by ivosidenib + venetoclax*	5 months / PD		Day 70	n/a	
4	13.8/M	*de novo* AML, CNS1	44%	NK	Second relapse post-HCT (in CR1), CNS1	33%	Complex[4]	none	250	Gr. 3: A	NR	no	no
	White, non-Hispanic			*WT1*, *RUNX1*, *FLT3* TKD			*WT1*, *RUNX1*	*HU on Day 33 of IDHi*	3 months / alternative therapy	Gr. 4: T, N	Day 87	n/a	

M: male, F: female, CNS: central nervous system, AML: acute myeloid leukemia, MDS: myelodysplastic syndrome, VAF: variant allele frequency, HCT: hematopoietic stem cell transplant, IDHi: IDH inhibitor, CR: complete remission, NK: normal karyotype, HMA: hypomethylating agent, GO: gemtuzumab ozogamicin, ADC: antibody drug conjugate, HU: hydroxyurea, ICU: intensive care unit, PD: progressive disease, IDH-DS: IDH differentiation syndrome, NR: non responder, PR: partial response, CRi: complete remission, incomplete count recovery, MRD: minimal residual disease, Gr.: Grade

[1]45,XY,add(6)(q25),der(7)t(7;13)(q32;q34),t(8;21)(q22;q22) [17]; 46,XX[3]; [2] thiotepa/vinorelbine/topotecan/clofarabine; mitoxantrone/cytarabine; [3] azacitidine, fludarabine, cytarabine, idarubicin; [4] 46,XY,del(15)(q15q21)[5]; 46,sl,add(13)(p11)[7]; 46,XY[7]//46,XX[3]

Table 2: Enasidenib therapy including patient and disease characteristics with treatment details and outcomes

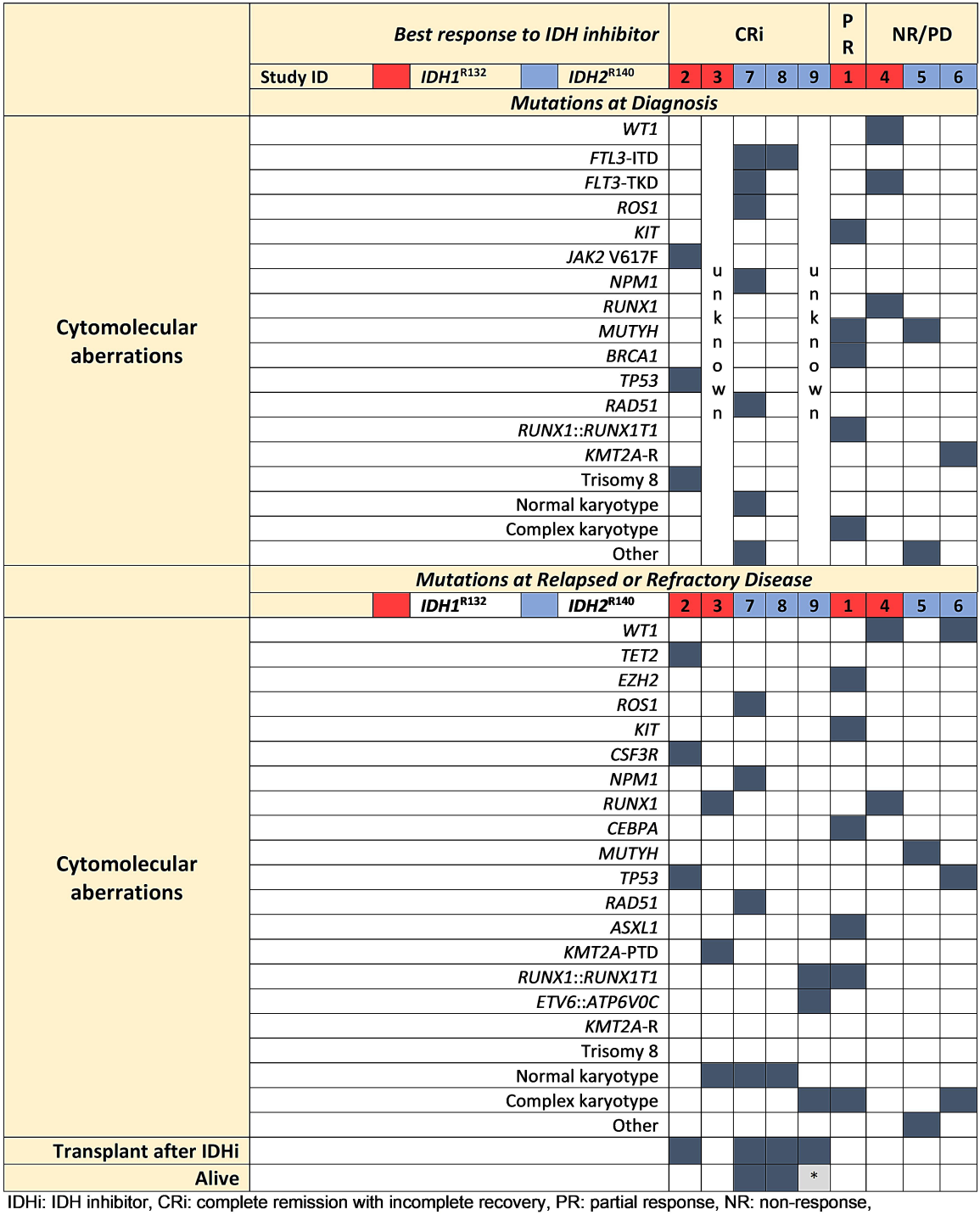
ID	Age (years) at IDHi use / Sex	At diagnosis			At time of IDH inhibitor use					Toxicity T=thrombo-cytopenia A=anemia N=neutropenia	Best response IDH VAF	HCT	Alive
	Race, ethnicity	Diagnosis, CNS status	*IDH2* VAF	*Karyotype* *Fusions, somatic mutations*	Disease state, CNS status	*IDH2* VAF	*Karyotype* *Fusions, somatic mutations*	Treatment before IDHi *with IDHi*	IDHi dose (mg) IDHi duration / reason for discontinuation		Time to best response	Post-HCT IDHi	
5	1.6/M White, Hispanic	*de novo* AMKL, CNS1, EMD^{pos} (chloroma)	VAF unknown	46,XY, der(21)t(1;21)(q25;q22) *MUTYH* G382D	Primary refractory, CNS1, EMD^{neg}	VAF unknown	Not repeated from diagnosis Not reported	Chemotherapy[5] *HMA, HU*	100 1 month / PD	Gr. 3: A Gr. 4: T, N	NR PD on Day 6	no n/a	no
6	7.8/M White, Hispanic	*de novo* AML, CNS2	mutation status unknown	Not reported *KTM2Ar* (9;11)	Post-HCT (in CR1) relapse (early^), CNS1	VAF unknown	Complex[6] *TP53*^{A276S}, *WT1*^{E207}, P124fs*34	Multiple lines of salvage[7] *none*	50 1.5 months / PD	Gr. 3: A Gr. 4: T, N	NR PD after 1.5 cycles	no n/a	no
7	8.3/F Black, non-Hispanic	*de novo* AML, CNS2a	VAF unknown	NK *NPM1, FLT3*^{N676K}, *FLT3* ITD (AR: 0.18), *RAD21*^{R437W}, *ROS1*^{R317W}	Post-chemo relapse (late^^), CNS1	VAF unknown	NK *NPM1*; *RAD21*^{R437W}, *ROS1*^{R317W}	Chemotherapy[8] + venetoclax *Chemotherapy*[9]	50 5 weeks pre-HCT / HCT	Gr. 3: A Gr. 4: T, N	CRi, MRD^{neg} VAF neg End of Cycle 1	yes no	yes
8	16.1/F Asian	*de novo* AML, CNS3	37%	47, XX, +11 [20] *FLT3* ITD (AR: 0.11)	Post-chemo relapse (early^), CNS1	40.6%	NK None identified	None *HMA, venetoclax*	100 pre-HCT 50 post-HCT 1 month / HCT Started 4.5 months post-HCT for 2 years	Gr. 3: A Gr. 4: T	CRi, MRD^{neg} VAF neg Day 81	yes yes	yes
9	18.9/M Other, non-Hispanic	*de novo* AML unknown CNS	mutation status unknown	Not reported Not reported	Post-HCT (in CR2) relapse (late^^), CNS1	43.6%	Complex[10] *RUNX1*::*RUNX1T1* *ETV6*::*ATP6V0C*	HMA, venetoclax *Chemotherapy*[1] *+/- venetoclax*	100 2.4 months pre-HCT Started 1.5 months post-HCT for 36 days / CMV viremia	Gr. 3: A Gr. 4: T, N	CRi, MRD^{neg} VAF neg Day 78	yes yes	no, NRM

M: male, F: female, CNS: central nervous system, AMKL: acute megakaryoblastic leukemia, EMD: extramedullary disease, AML: acute myeloid leukemia, VAF: variant allele frequency, HCT: hematopoietic stem cell transplant, IDHi: IDH inhibitor, CR: complete remission, NK: normal karyotype, HMA: hypomethylating agent, HU: hydroxyurea, PD: progressive disease, CMV: cytomegalovirus, Gr.: Grade, NR: non responder, CRi: complete remission with incomplete count recovery, MRD: minimal residual disease, NRM: non-relapse mortality, ^early relapse <1 year from diagnosis, ^^late relapse >1 year from diagnosis.
[5] 2 cycles on ATACC AML (AAML1031 backbone with atovaquone starting on Induction day 6), 2 cycles TACL2016-003 (single arm, decitabine/vorinostat/FLAG), CPX-351; [6] 46,XY,t(1;3)(q?21;p?21) [1]; t(2;8)(p?24;p?12),add(9)(p21),der(11)add(11)(p15)ins(11;9)(q23;p21p21, ?del(17)(p12p12)[18]; 45,sl,der(9)t(9;9)(q34;q22),der(9)add(9)(p22)t(9;9),t(13;14)(q32;q22),-15,der(18), t(15;18)(q11.2;p11.2) [2]; [7] (1): azacitidine/gemtuzumab; (2): panobinostat/fludarabine/cytarabine (PANAML, St. Jude); (3): selinexor/fludarabine/cytarabine (SELHEM, St. Jude); (4): MTX/asparaginase; (5): CPX-351; (6): decitabine/vorinostat 230 mg/m2; [8] CPX-351 + venetoclax; fludarabine/cytarabine/granulocyte stimulating colony factor (GCSF); [9] fludarabine/cytarabine/GCSF ; [10] 46,XY,add(3)(q21),add(7)(q32),?add(8)(q24),add(9)(q22),add(21)(q22)[5]; 46,sl,t(1;12)(q21;q24.1),?add(22)(q11.2)[3]; [11] CPX-351 + venetoclax; fludarabine/cytarabine/GCSF

Figure Legend

Figure 1. Somatic mutational landscape at diagnosis and at relapsed/refractory timepoint and treatment response to IDH inhibitor. IDHi: IDH inhibitor, CRi: complete remission with incomplete recovery, PR: partial response, NR: non-response, PD: progressive disease; ITD: internal tandem duplication, TKD: tyrosine kinase domain, * death from non-relapse mortality

Figure 1. Somatic mutational landscape at diagnosis and at relapsed/refractory timepoint and treatment response to IDH inhibitor



IDHi: IDH inhibitor, CRi: complete remission with incomplete recovery, PR: partial response, NR: non-response, PD: progressive disease; ITD: internal tandem duplication, TKD: tyrosine kinase domain, * death from non-relapse mortality