



Addendum to the FDA Briefing Document
Pediatric Oncology Subcommittee of the
Oncologic Drugs Advisory Committee Meeting (ODAC)
June 20, 2018

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The following set of slides on the Candidate Molecular Target List will be added to the Briefing Document. Please see the next page.

Candidate Molecular Target List

Primary Target Class

- Gene Abnormality
- Cell Lineage
- Tumor Microenvironment & Immunotherapy
- Others
- Automatic Waivers

Gene Abnormality

Target Symbol	Gene Abnormality
ABL1/2	ABL1/2 gene fusions (BCR-ABL1, etc.)
ACVR1	ACVR1
ALK	ALK and ALK gene fusions
ASCL1	ASCL1 gene
BRAF	BRAF
CDK12	EWSR1-FLI1
CSF1R	CSF1R gene fusions
CTNNB1 (β-catenin)	CTNNB1
DDX3X	DDX3X
DOT1L	MLL gene fusions
EGFR	EGFR
ERK	BRAF, MAP2K1

Target Symbol	Gene Abnormality
EWSR1-FLI1	EWSR1-FLI1
EZH2	SMARCB1, SMARCA4
FGFR	FGFR and FGFR gene fusions
FLT3	FLK2, STK1, CD135
Gamma secretase	NOTCH1 and FBXW7
GFI1	GFI1
GFI1B	GFI1B
Histone 3 G34R/V	Histone 3 G34R/V
Histone 3 K27M	Histone 3 K27M
IDH1 and IDH2	IDH1 and IDH2
JAK1, 2, and 3	JAK1, 2, and 3
LIN28B	LIN28B

Gene Abnormality

Target Symbol	Gene Abnormality
MDM2	MDM2, TP53
MEK	BRAF and BRAF gene fusions, MAP2K1, NF1
Menin	MLL gene fusions
MET	MET
MLL	MLL gene fusions
mTOR	TSC1, TSC2
MYC	MYC translocations and amplification
MYCN	MYCN amplification
Neoantigens	MSH2, MLH1, MSH6, PMS2 POLE, and POLD1
NFkappaB	RELA fusion
NOTCH1	NOTCH1, FBXW7
NT5C2	NT5C2

Target Symbol	Gene Abnormality
NTRK	NTRK gene fusions
ODC1	MYC target gene
PARP	BRCA1/2, PALB2, ATM, BRIP1, CHEK2, RAD51, etc.
PAX-FOXO1	PAX-FOXO1
PDGFRA/B	PDGFRA/B gene fusions
PI3K α	PIK3CA
PPM1D (WIP1)	PPM1D (WIP1)
RAS	RAS
RET	RET
SH2B3	SH2B3
SHP2	SHP2
Smoothened	PATCH1, SMO

Gene Abnormality

Target Symbol	Gene Abnormality
SYT-SSX	SYT-SSX
TERT	TERT
TORC1/2 as distinct from mTOR	TORC1/2
TrkB	TrkB
TP53	TP53
TYK2	TYK2

Cell Lineage

Target Symbol (1)	Target Symbol (2)	Target Symbol (3)	Target Symbol (4)
AKR1C3	CD70	GPNMB	
BCOR	CD79b	ERBB2 (HER2/Neu)	SYK
BTK	CD123/IL3RA	IL6	
CD7	CD276 (B7-H3)		WT1
CD19	Cereblon CBL (E3 Ubiquitine protein ligase)	IL13RA2	
CD20	DLL3	LRRC15	YAP1
CD22	DLK1	MAGE-A3	
CD30	EGFRvIII	MSLN (mesothelin)	
CD33	EPHA2	NR5A1 (Steroidogenic factor-1)	
CD37	GD2	NY-ESO-1	
CD38	GPC2	Olig2	
CD56	GPC3	PIK3CD (PI3 kinase delta)	
		PRAME	

Tumor Microenvironment & Immunotherapy



Target Symbol (1)
B7H3
CD40
CD47
CD52
CXCR4
CXCR10
CTLA4
GM-CSF
IDO1
IFN-gamma
IL-2
LAG3

Target Symbol (2)
OX40
PD-1/PD-L1
RELA
RIG-I
STEAP1
STING
TIM3/TIM4

Others

Target Symbol (1)	Target Symbol (2)	Target Symbol (3)	Target Symbol (4)
AKT	BMPR	DNA-PK	LSD1
ATM	Brd1	DNMT (DNA methyl transferase)	MCL1
ATR	Brd4	FAK	MCT1 (monocarboxyate transporter 1)
ATRX	CDK4/6	FOLR1 (folate receptor 1)	MEK
AURKA (Aurora kinase A)	CHK1	GSK-3	MIZ1
AURKB (Aurora kinase B)	CDK2	HDAC	MGMT
AXL	CDK7	HIF1A	MLL5
	CDK9	Hippo pathway (YAP, TAZ, TEADs)	MYST3 (MYST histone acetyltransferase (monocytic leukemia))
A1/BFL	CK1		
BAK	CK2 (casein kinase 2)	Hsp90	NAMPT
BAX	CREBBP/EP300	IAPs (inhibitor-of-apoptosis)	NEDD8 activating enzyme (NAE)
BCL2 family members (Bcl-2, Bcl-XL, Mcl-1, A1/BFL, BAK, BAX)	DNA (alkylators)	IGFR-1	PARP
BET bromodomain family		KDM4A	PDK-1 (3-phosphoinositide-dependent protein kinase 1)

Others

Target Symbol (5)

PI3Kdelta

PIM1

PKA

PKC

PLK1

POL1

PRDM1

PRDM8

PRDM10

PRMT2

PRMT5

Proteasome

Target Symbol (6)

TRAIL

Tubulin

XPO1 (Exportin)

WDR5

WEE1

Automatic Waivers

Target Symbol
AR
ESR1
ESR2
GnRHR
PSA
PSCA
PSMA
VEGF
VEGFR

