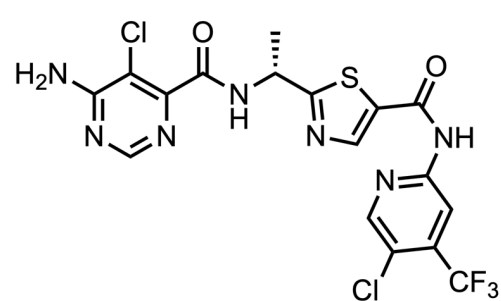
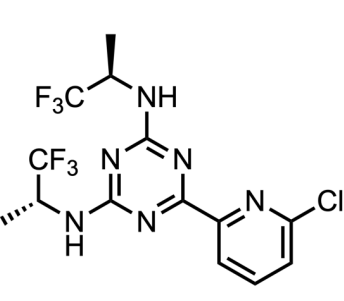


2024 Novel Small Molecule FDA Drug Approvals

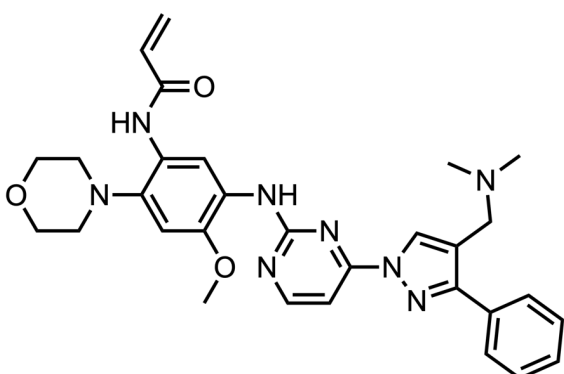
Oncology (6)



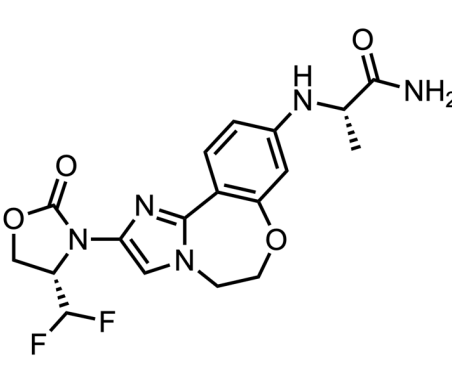
tovorafenib
(Ojemda)^a
type II RAF kinase inhibitor
for R/R pediatric LGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation
125 - 600 mg PO Q1W
DAY ONE (SUNESIS)



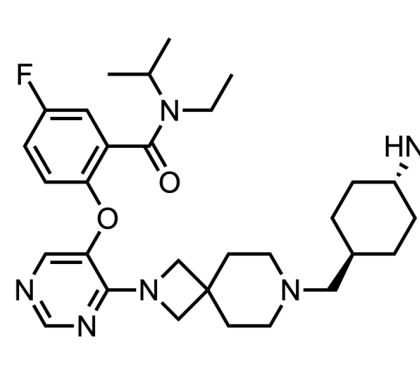
vorasidenib
(Vorango)
IDH1/IDH2 inhibitor
for Grade 2 astrocytoma or oligodendroglioma with a susceptible IDH1 or IDH2 mutation
20 - 40 mg PO QD
SERVIER (AGIOS)



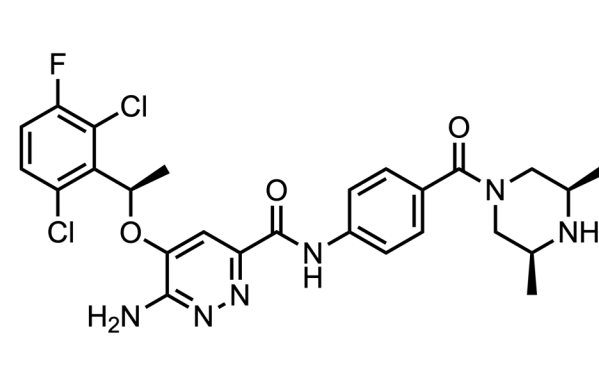
lazertinib
(Lazluze)
mutant-selective EGFR inhibitor
for non-small cell lung cancer
240 mg PO QD
JANSSEN (GENOSCO/OSCOTEC)



inavolisib
(Itovebi)
PI3K α inhibitor and degrader
for endocrine-resistant, *PIK3CA* mutated, HR+/HER2-breast cancer
9 mg PO QD
GENENTECH

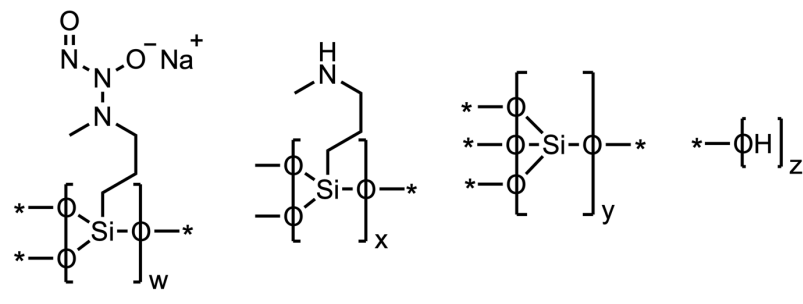


revumenib
(Revuforj)^b
menin-KMT2A PPI inhibitor
for R/R acute leukemia with a KMT2A translocation
160 - 270 mg PO BID
SYNTAX (VITAE)

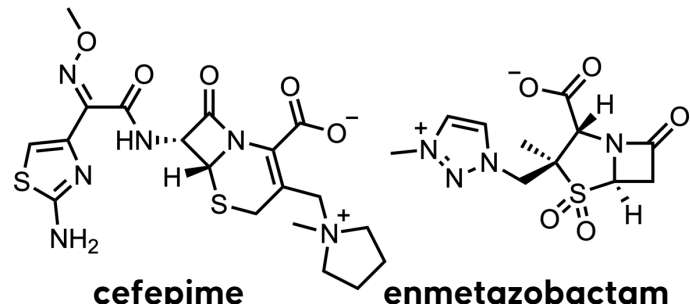


ensartinib
(Ensacove)
ALK inhibitor
for ALK-positive non-small cell lung cancer
225 mg PO QD
XCOVERY

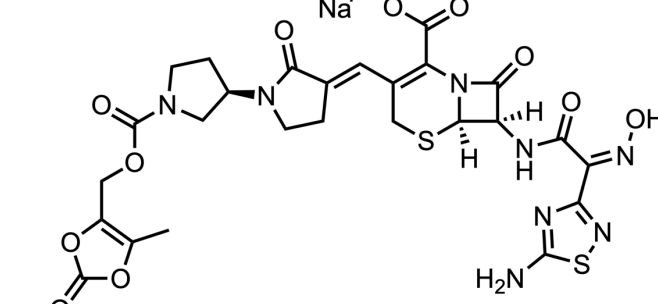
Infectious Diseases (4)



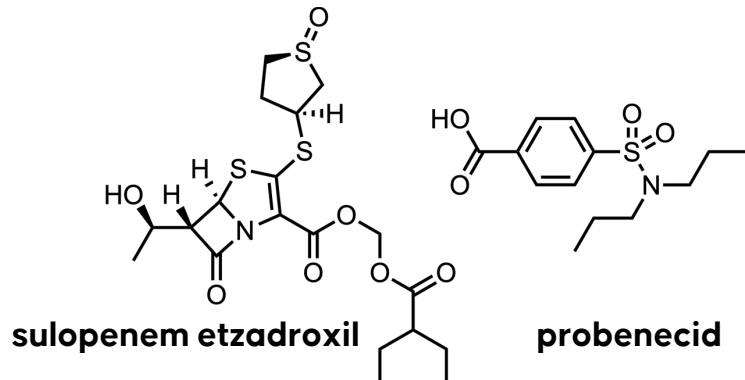
berdazimer
(Zelsuvmi)^b
nitric oxide releasing agent^c
for molluscum contagiosum
0.5 mL topical QD
PELTHOS (UNC-CHAPEL HILL / NOVAN)



cefepime, enmetazobactam
(Exblifep)
cefepime: cell wall synthesis inhibitor
enmetazobactam: beta-lactamase inhibitor
for complicated urinary tract infections
2.5 g IV infusion TID
ALLECRA (ORCHID)

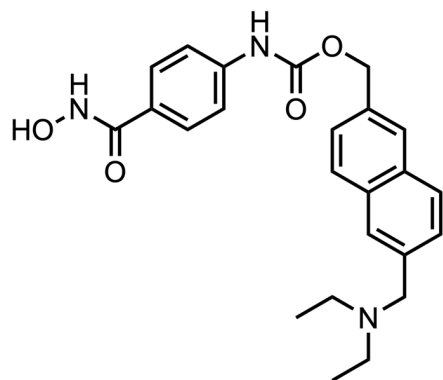


ceftobiprole medocartil sodium
(Zevtera)
cell wall synthesis inhibitor
for SAB, ABSSSI, CABP
667 mg IV infusion TID or QID
BASILEA PHARMAEUTICA (ROCHE)

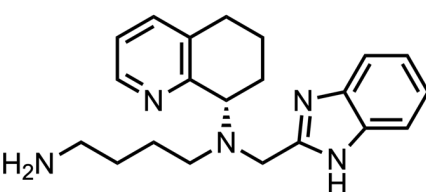


sulopenem etzadroxil, probenecid
(Orlynvah)
sulopenem: cell wall synthesis inhibitor
probenecid: OAT3 inhibitor
for uncomplicated urinary tract infections in women
500 mg/500 mg PO BID
ITERUM (PFIZER)

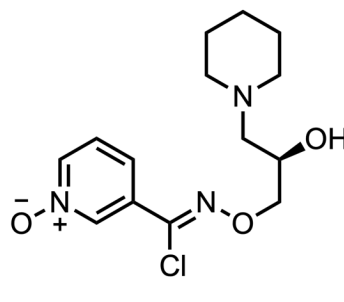
Rare / Genetic Diseases (6)



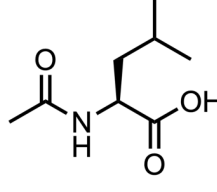
givinostat
(Duvyzat)^b
HDAC inhibitor
for Duchenne muscular dystrophy
22.2 - 53.2 mg PO BID
ITF THERAPEUTICS (ITALFARMACO)



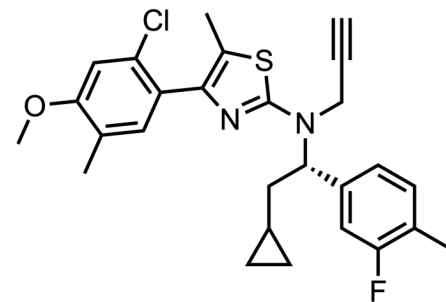
mavorixafor
(Xolremdi)^b
CXCR4 antagonist
for WHIM syndrome
300 - 400 mg PO QD
X4 PHARMACEUTICALS (ANORMED / GENZYME)



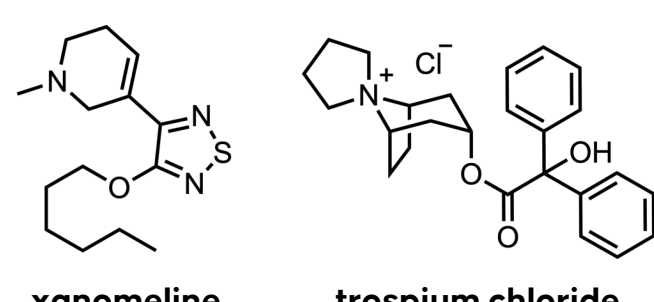
arimoclomol
(Miplyffa)^b
unknown MoA
for Niemann-Pick disease type C
47 - 124 mg PO TID
ZEVRA (ORPHAZYME)



levacetylleucine
(Aqneursa)^b
unknown MoA
for Niemann-Pick disease type C
schedule varies by patient body weight - oral administration
INTRABIO (UNIVERSITY OF OXFORD / UNIVERSITY OF MUNICH)

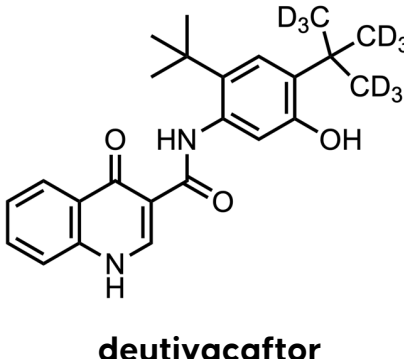
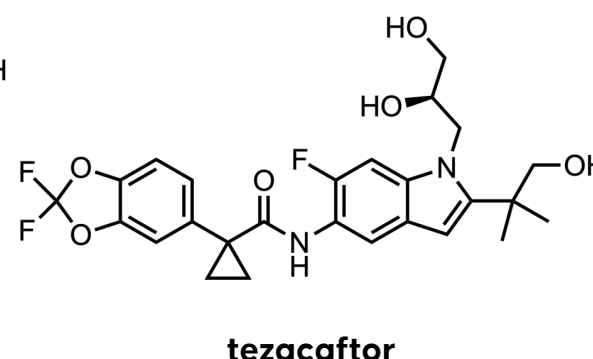
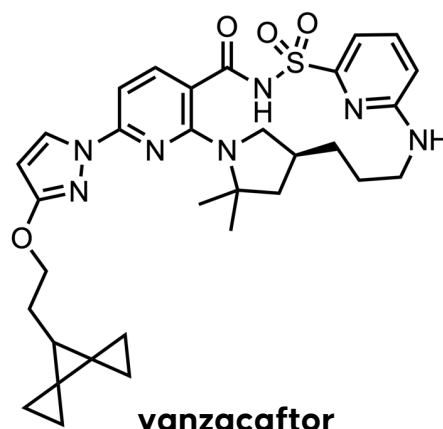


crinecerfont
(Crenessity)^b
corticotropin-releasing factor type 1 receptor antagonist
for classic congenital adrenal hyperplasia
25 - 100 mg PO BID
NEUROCRINE (SANOFI-SYNTHELABO)

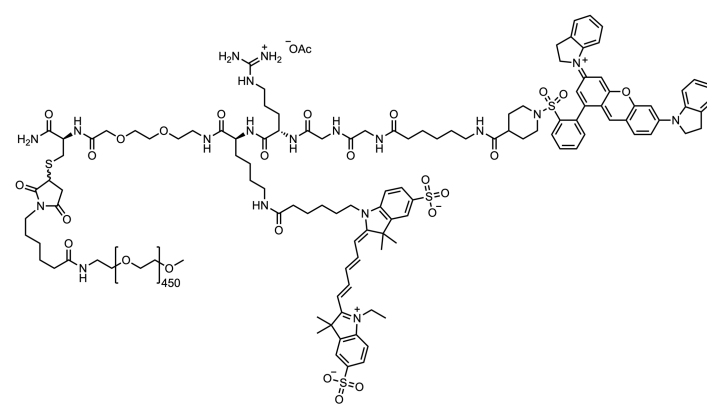


xanomeline and trospium chloride
(Cobenfy)^b
xanomeline: M1/M4 muscarinic acetylcholine receptor agonist
trospium chloride: peripheral muscarinic antagonist
for schizophrenia
50/20 - 125/30 mg PO BID
BRISTOL MYERS SQUIBB (KARUNA)

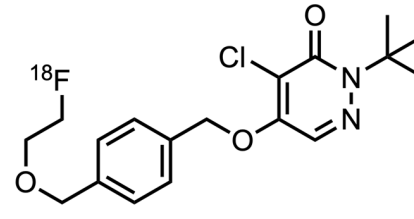
Rare / Genetic (cont)



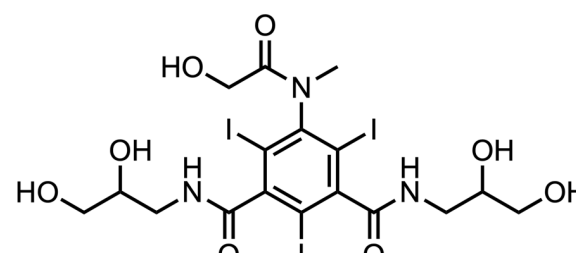
vanzacaftor, tezacaftor, deutivacaftor
(Alyftrek)
vanzacaftor, tezacaftor: CFTR correctors
deutivacaftor: CFTR potentiator
for cystic fibrosis
4/20/50 mg PO TID or 10/50/125 mg PO BID
VERTEX



pegulicianine
(Lumisight)^b
becomes optically active upon peptide chain cleavage by cathepsins and matrix metalloproteases
for fluorescence imaging in adults with breast cancer
1 mg/kg IV 2 - 6 h prior to imaging
LUMICELL

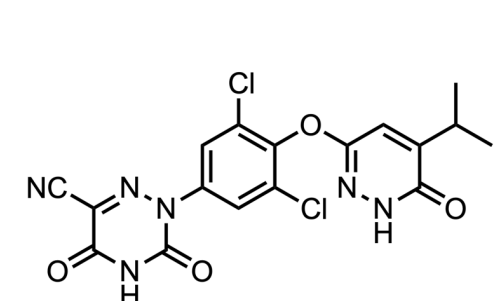


flurpiridaz F 18
(Flyrcado)
mitochondrial complex 1 inhibitor^d
for evaluation of myocardial ischemia and infarction
93 - 352 MBq IV
GE HEALTHCARE (BMS MEDICAL IMAGING / LANTHEUS MEDICAL IMAGING)

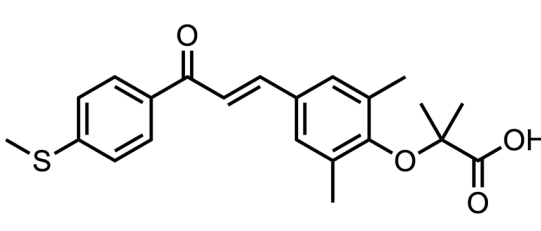


iomeprol
(Iomervu)
radiographic iodinated contrast agent
for radiographic visualization of the internal structures
dose is optimized based on age, body weight, vessel size, rate of blood flow within the vessel, and structures or areas to be examined
BRACCO DIAGNOSTICS

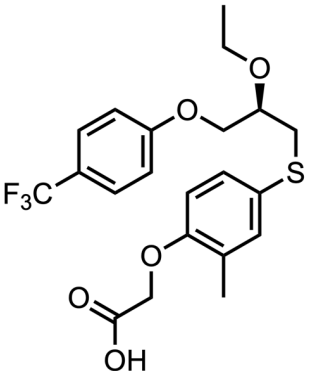
Hepatology (3)



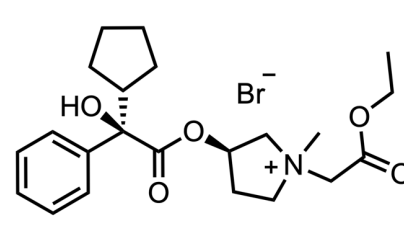
resmetirom
(Rezdiffra)^{a,b}
THR- β partial agonist
for NASH with moderate to advanced liver fibrosis
80 - 100 mg PO QD
MADRIGAL (ROCHE)



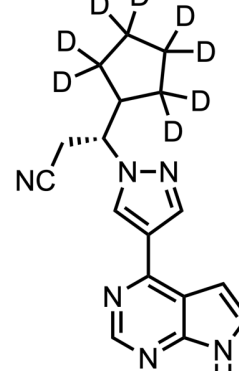
elafibranor
(Iqirvo)^{a,b}
PPAR α / δ agonist
for primary biliary cholangitis
80 mg PO QD
IPSEN (GENFIT)



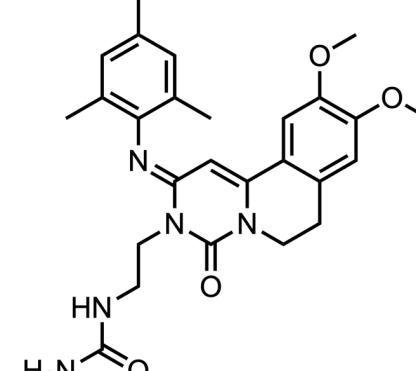
seladelpar
(Livdelzi)^a
PPAR δ agonist
for primary biliary cholangitis
10 mg PO QD
GILEAD (JANSSEN)



sofipironium bromide
(Sofdra)
M3 muscarinic acetylcholine receptor antagonist
for primary axillary hyperhidrosis
72 mg topically QD
BOTANIX (BODOR LABORATORIES)

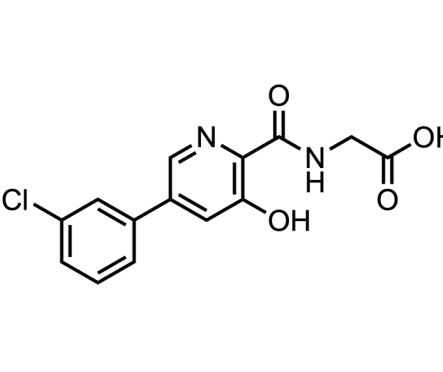


deuruxolitinib
(Leqselvi)
JAK inhibitor
for severe alopecia areata
8 mg PO BID
SUN PHARMA (CONCERT)

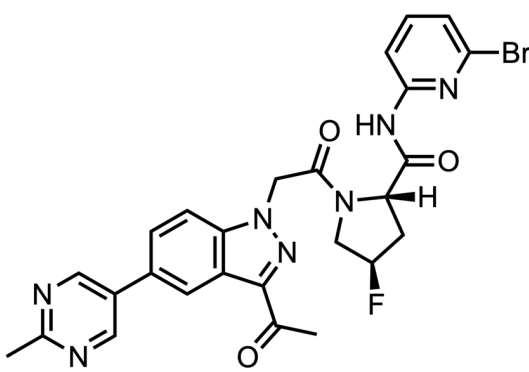


ensifentrine
(Ohtuvayre)
PDE3/4 inhibitor
for chronic obstructive pulmonary disease
3 mg inhalation BID
VERONA (VERNALIS)

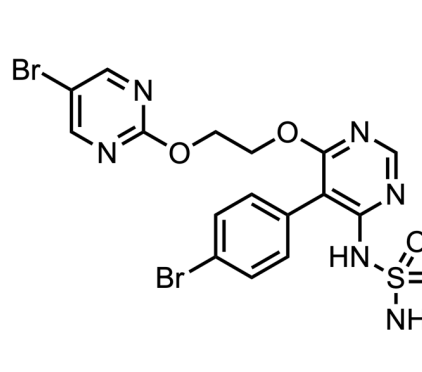
Hematology (2)



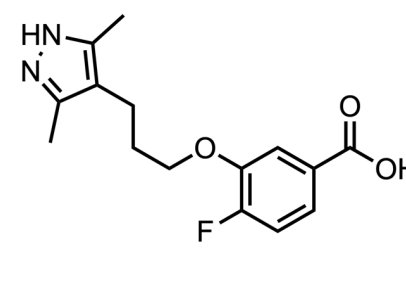
vadadustat
(Vafseo)
HIF PH inhibitor
for anemia due to chronic kidney disease
150 - 600 mg PO QD
AKEBIA (PROCTER & GAMBLE)



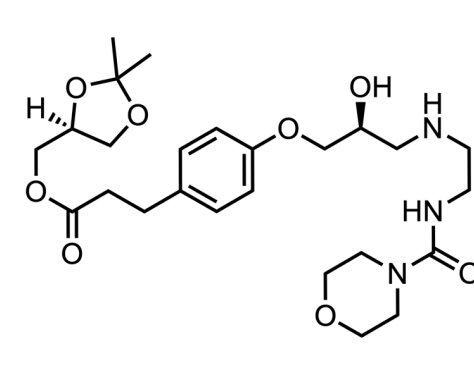
danicopan
(Voydeya)^b
complement factor D inhibitor
for extravascular hemolysis in paroxysmal nocturnal hemoglobinuria
150 - 200 mg PO TID
ALEXION (ACHILLION)



aprocitentan
(Tryvio)^b
dual ET_A and ET_B receptor antagonist
for hypertension
12.5 mg PO QD
IDORSIA (ACTELION)



acoramidis
(Attruby)
TTR stabilizer
WT or variant transthyretin amyloid cardiomyopathy
712 mg PO BID
BRIDGEBIO (STANFORD UNIVERSITY)



landiolol
(Rapiblyk)
 β 1-adrenoceptor antagonist
for supraventricular tachycardia IV infusion titrated according to ventricular rate
AOP ORPHAN (ONO PHARMA)

Footnotes

^a accelerated approval
^b first-in-class
^c the mechanism of action for the treatment of molluscum contagiosum is unknown
^d binds to heart tissue that has biologically active mitochondria

Abbreviations:
ABSSSI: acute bacterial skin and skin structure infections, ALK: anaplastic lymphoma kinase, CABP: community-acquired bacterial pneumonia, HIF PH: hypoxia-inducible factor prolyl hydroxylase, LGG: low-grade glioma, NASH: noncirrhotic non-alcoholic steatohepatitis, PPI: protein-protein interaction, R/R: relapsed or refractory, SAB: *Staphylococcus aureus* bloodstream infections, WHIM: Warts, Hypogammaglobulinemia, Infections, and Myelokathexis,

The reported doses are general guidelines and may not apply to special populations. This poster is for informational purposes only and is not intended for medical use.