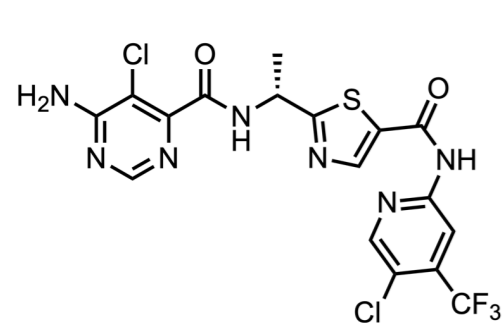
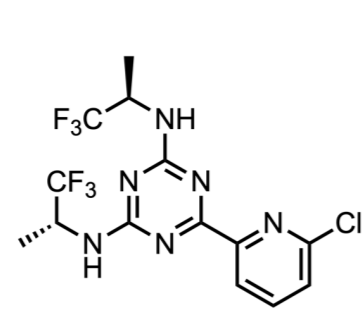


# 2024 Novel Small Molecule FDA Drug Approvals

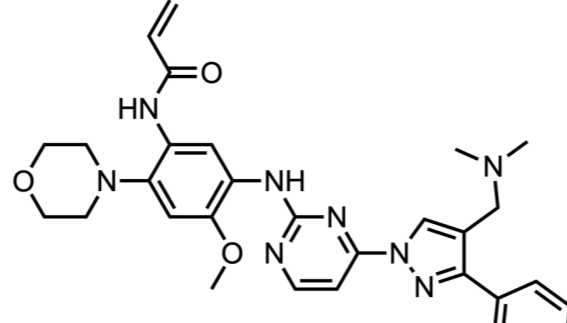
## Oncology (6)



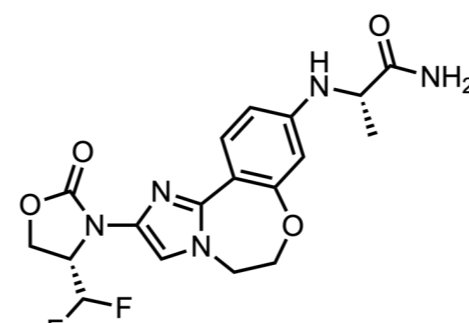
**tovorafenib**  
(Ojemda)<sup>a</sup>  
**type II RAF kinase inhibitor**  
for R/R pediatric LGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation  
125 - 600 mg PO Q1W  
DAY ONE (SUNESIS)



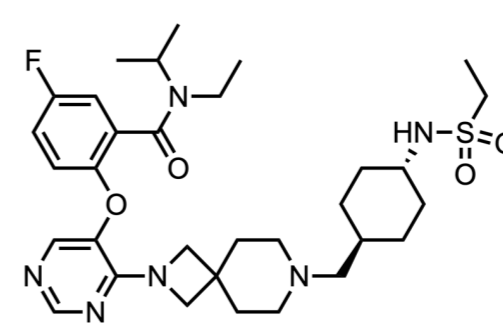
**vorasidenib**  
(Vorango)  
**IDH1/IDH2 inhibitor**  
for Grade 2 astrocytoma or oligodendroglioma with a susceptible IDH1 or IDH2 mutation  
20 - 40 mg PO QD  
SERVIER (AGIOS)



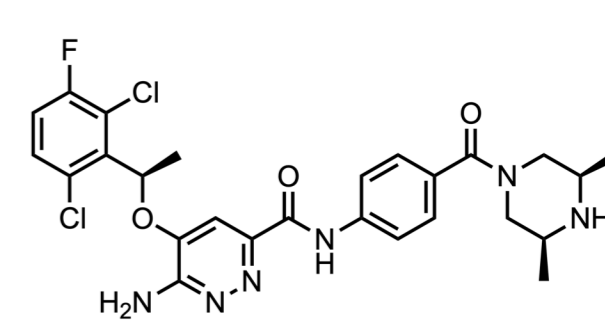
**lazertinib**  
(Lazluze)  
**mutant-selective EGFR inhibitor**  
for non-small cell lung cancer  
240 mg PO QD  
JANSSEN (GENOSCO/OSCOTEC)



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

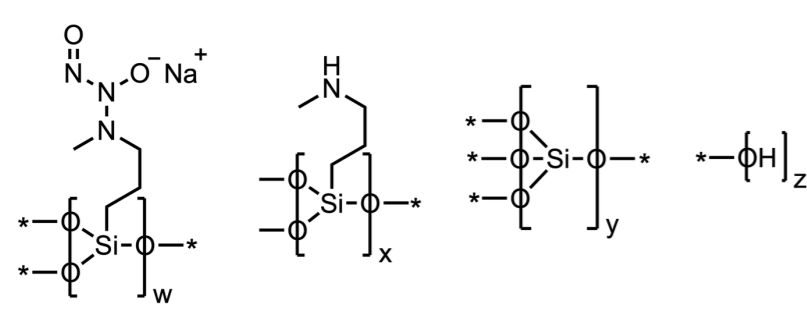


**revumenib**  
(Revuforj)<sup>b</sup>  
**menin-KMT2A PPI inhibitor**  
for R/R acute leukemia with a KMT2A translocation  
160 - 270 mg PO BID  
SYNTAX (VITAE)

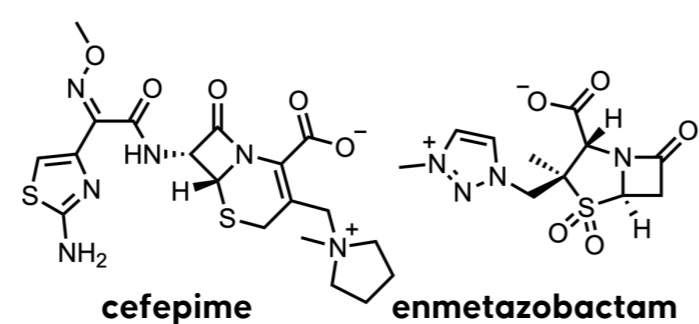


**ensartinib**  
(Ensacove)  
**ALK inhibitor**  
for ALK-positive non-small cell lung cancer  
225 mg PO QD  
XCOVERY

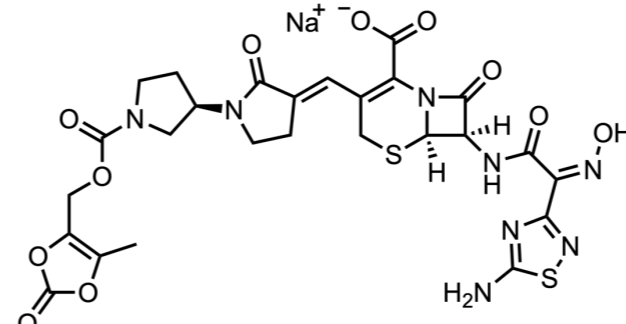
## Infectious Diseases (4)



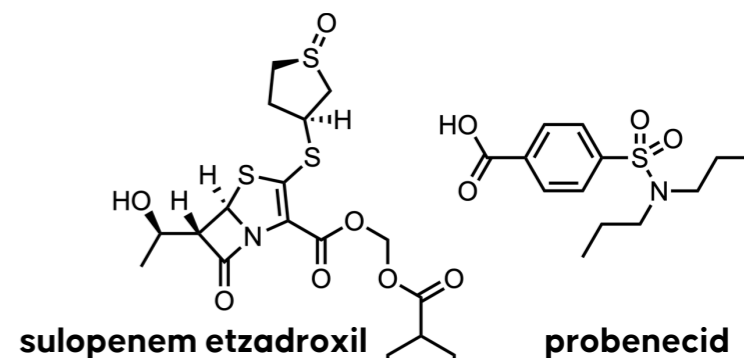
**berdazimer**  
(Zelsuvmi)<sup>b</sup>  
**nitric oxide releasing agent<sup>c</sup>**  
for molluscum contagiosum  
0.5 mL topical QD  
PELTHOS (UNC-CHAPEL HILL / NOVAN)



**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)

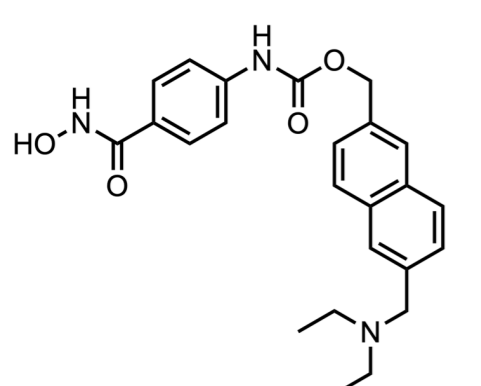


**ceftobiprole medocaril sodium**  
(Zevtera)  
**cell wall synthesis inhibitor**  
for SAB, ABSSSI, CABP  
667 mg IV infusion TID or QID  
BASILEA PHARMAEUTICA (ROCHE)

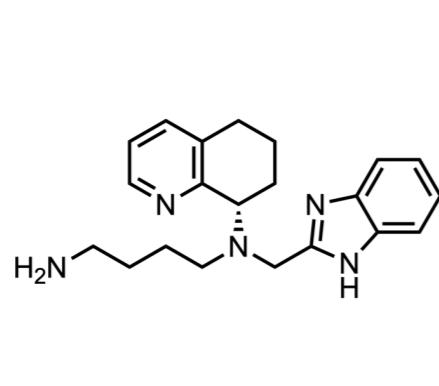


**sulopenem etzadroxil, probenecid**  
(Orlynvah)  
**sulopenem: cell wall synthesis inhibitor**  
**probenecid: OAT3 inhibitor**  
for uncomplicated urinary tract infections in women  
500 mg/500 mg PO BID  
ITERUM (PFIZER)

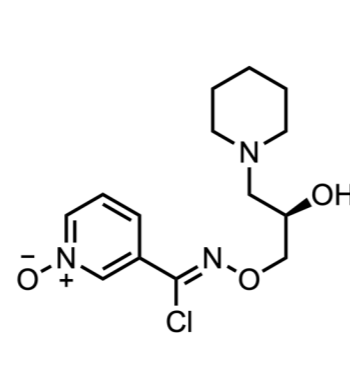
## Rare / Genetic Diseases (6)



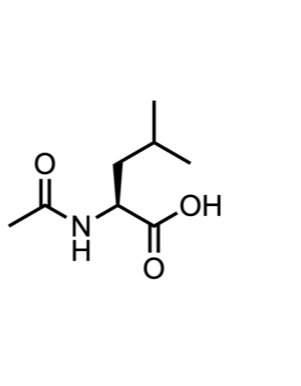
**givinostat**  
(Duvyzat)<sup>b</sup>  
**HDAC inhibitor**  
for Duchenne muscular dystrophy  
22.2 - 53.2 mg PO BID  
ITF THERAPEUTICS (ITALFARMACO)



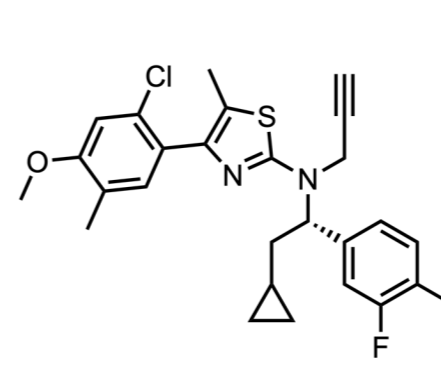
**mavorixafor**  
(Xolremdi)<sup>b</sup>  
**CXCR4 antagonist**  
for WHIM syndrome  
300 - 400 mg PO QD  
X4 PHARMACEUTICALS (ANORMED / GENZYME)



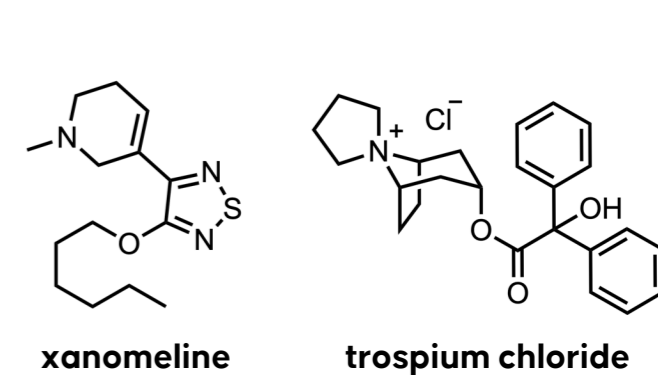
**arimocloamol**  
(Miplyffa)<sup>b</sup>  
**unknown MoA**  
for Niemann-Pick disease type C  
47 - 124 mg PO TID  
ZEVRA (ORPHAZYME)



**levacetylleucine**  
(Aqeursu)<sup>b</sup>  
**unknown MoA**  
for Niemann-Pick disease type C  
schedule varies by patient body weight - oral administration  
INTRABIO (UNIVERSITY OF OXFORD / UNIVERSITY OF MUNICH)

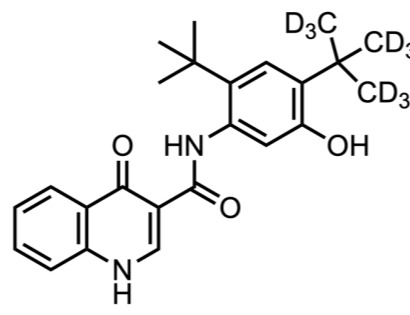
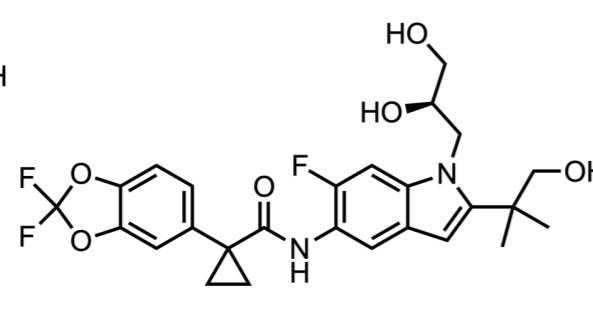
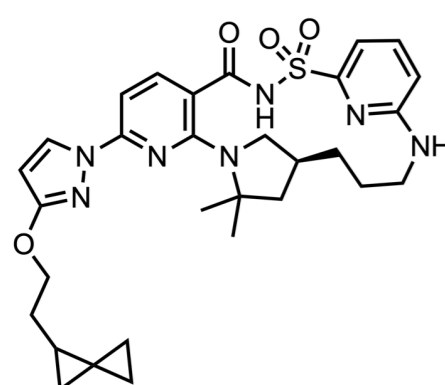


**crinercerfont**  
(Crenesity)<sup>b</sup>  
**corticotropin-releasing factor type 1 receptor antagonist**  
for classic congenital adrenal hyperplasia  
25 - 100 mg PO BID  
NEUROCRINE (SANOFI-SYNTHELABO)

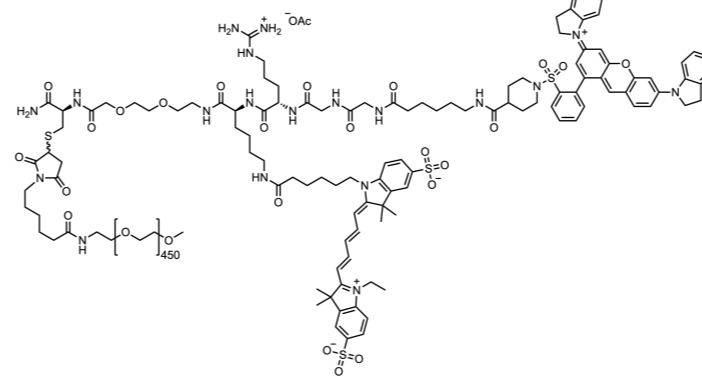


**xanomeline and trospium chloride**  
(Cobenty)<sup>b</sup>  
**xanomeline: M1/M4 muscarinic acetylcholine receptor agonist**  
**trospium chloride: peripheral muscarinic antagonist**  
for schizophrenia  
50/20 - 125/30 mg PO BID  
BRISTOL MYERS SQUIBB (KARUNA)

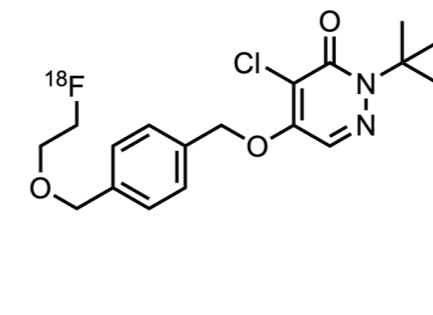
## Rare / Genetic (cont)



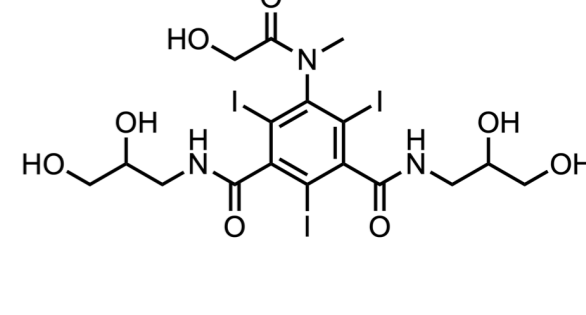
**vanzacaftor, tezacaftor, deutivacaftor**  
(Alyftrek)  
**vanzacaftor, tezacaftor: CFTR correctors**  
**deutivacaftor: CFTR potentiator**  
for cystic fibrosis  
4/20/50 mg PO TID or 10/50/125 mg PO BID  
VERTEX



**pegulicanine**  
(Lumisight)<sup>b</sup>  
**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

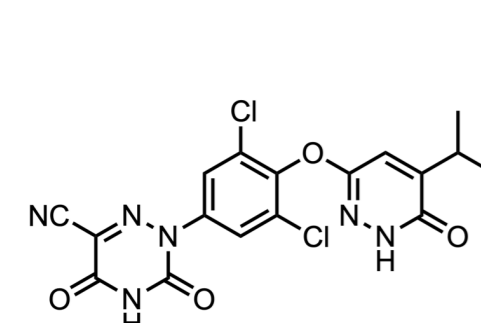


**flurpiridaz F 18**  
(Flyrcado)  
**mitochondrial complex 1 inhibitor<sup>d</sup>**  
for evaluation of myocardial ischemia and infarction  
93 - 352 MBq IV  
GE HEALTHCARE (BMS MEDICAL IMAGING / LANTHEUS MEDICAL IMAGING)

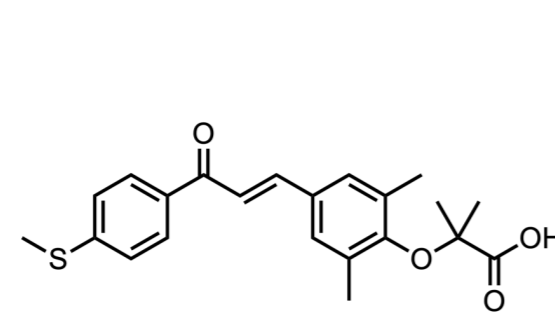


**iomeprol**  
(Iomervu)  
**radiographic iodinated contrast agent**  
for radiographic visualization of the internal structures  
dose is optimized based on age, body weight, vessel size, rate of blood flow within the vessel, and structures or areas to be examined  
BRACCO DIAGNOSTICS

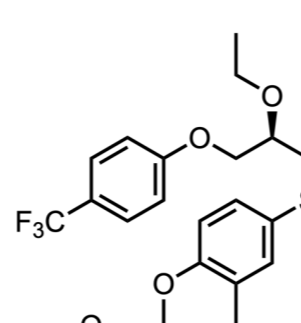
## Hepatology (3)



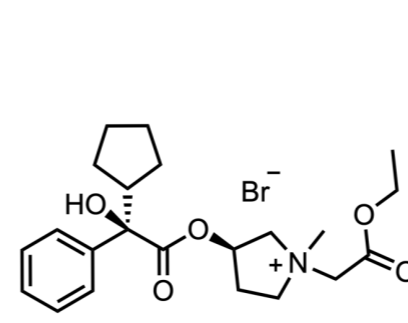
**resmetirom**  
(Rezdiffra)<sup>a,b</sup>  
**THR- $\beta$  partial agonist**  
for NASH with moderate to advanced liver fibrosis  
80 - 100 mg PO QD  
MADRIGAL (ROCHE)



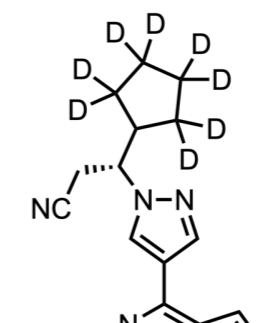
**elafibranor**  
(Iqirvo)<sup>a,b</sup>  
**PPAR $\alpha/\delta$  agonist**  
for primary biliary cholangitis  
80 mg PO QD  
IPSEN (GENFIT)



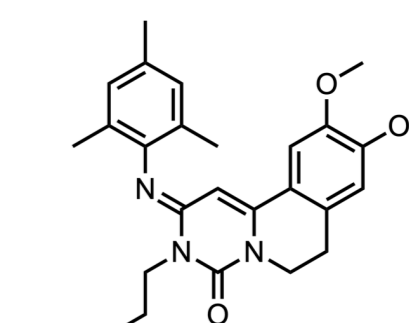
**seladelpar**  
(Livdelzi)<sup>a</sup>  
**PPAR $\delta$  agonist**  
for primary biliary cholangitis  
10 mg PO QD  
GILEAD (JANSSEN)



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

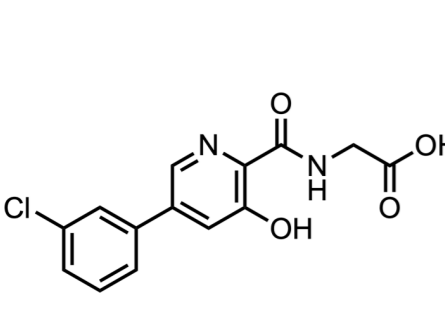


**deuruxolitinib**  
(Leqselvi)  
**JAK inhibitor**  
for severe alopecia areata  
8 mg PO BID  
SUN PHARMA (CONCERT)

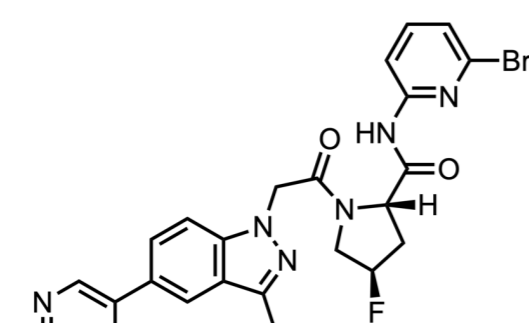


**ensifentrine**  
(Ohtuvayre)  
**PDE3/4 inhibitor**  
for chronic obstructive pulmonary disease  
3 mg inhalation BID  
VERONA (VERNALIS)

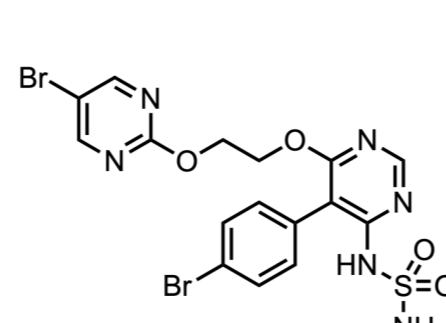
## Hematology (2)



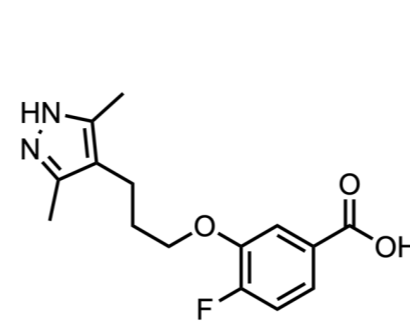
**vadadustat**  
(Vafseo)  
**HIF PH inhibitor**  
for anemia due to chronic kidney disease  
150 - 600 mg PO QD  
AKEBIA (PROCTER & GAMBLE)



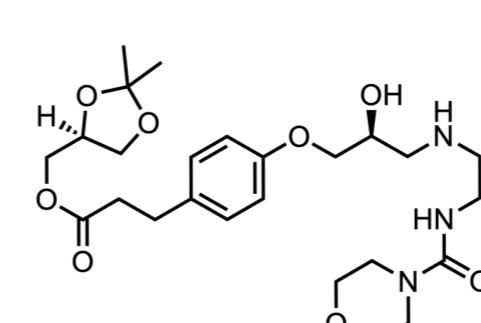
**danicopan**  
(Voydeya)<sup>b</sup>  
**complement factor D inhibitor**  
for paroxysmal nocturnal hemoglobinuria  
150 - 200 mg PO TID  
ALEXION (ACHILLION)



**aprocitentan**  
(Tryvio)<sup>b</sup>  
**dual ET<sub>A</sub> and ET<sub>B</sub> 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)  
 **$\beta$ 1-adrenoceptor antagonist**  
for supraventricular tachycardia IV infusion titrated according to ventricular rate  
AOP ORPHAN (ONO PHARMA)

## Footnotes

<sup>a</sup> accelerated approval  
<sup>b</sup> first-in-class  
<sup>c</sup> the mechanism of action for the treatment of molluscum contagiosum is unknown  
<sup>d</sup> 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.