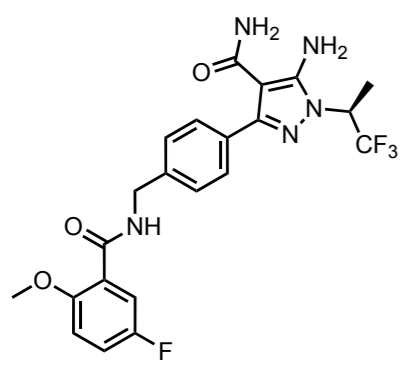


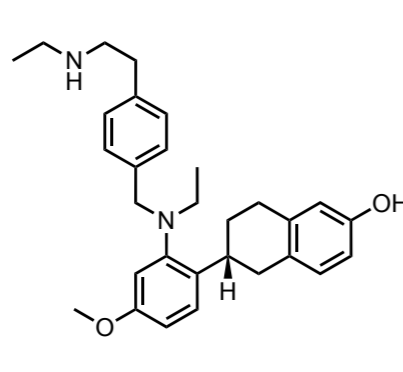
# 2023 Novel Small Molecule Drug Approvals

## Oncology (8)



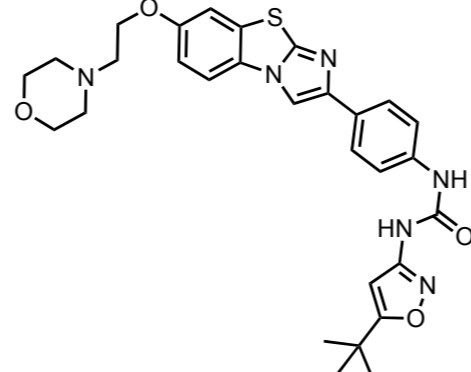
**pirtobrutinib**  
(Jaypirca)\*  
**BTK inhibitor**

for relapsed or refractory mantle cell lymphoma  
200 mg orally QD  
**REDX PHARMA (LOXO/LILLY)**



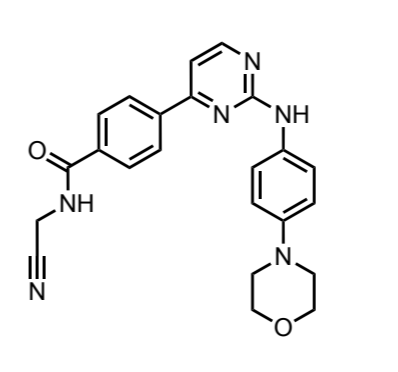
**elacestrant**  
(Orserdu)  
**SERD/SERM**

for ER+, HER2-, ESR1-mutated breast cancer  
345 mg orally QD  
**EISAI (STEMLINE)**



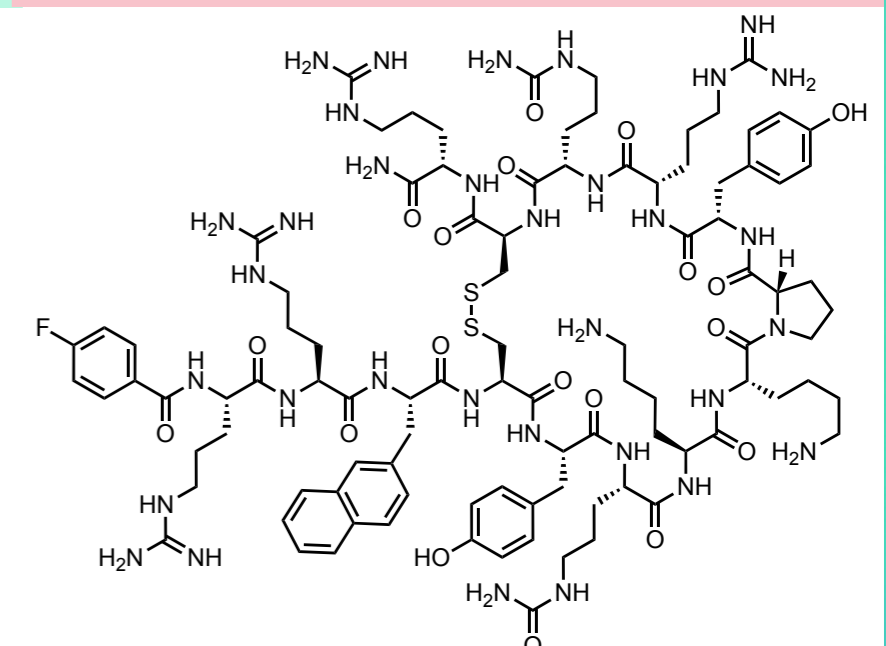
**quizartinib**  
(Vanflyta)  
**FLT3 inhibitor**

for acute myeloid leukemia  
35.4, 26.5 or 53 mg orally QD  
**AMBIT BIOSCIENCES (DAICHI SANKYO)**



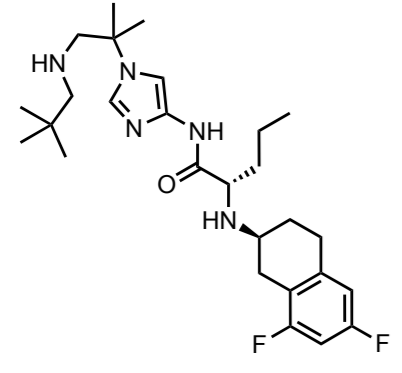
**momelotinib**  
(Ojjaara)  
**JAK1/2<sup>WT</sup>, JAK2<sup>V617F</sup>, ACVR1 inhibitor**

for myelofibrosis with anemia  
200 mg orally QD  
**CYTOPIA (GSK)**



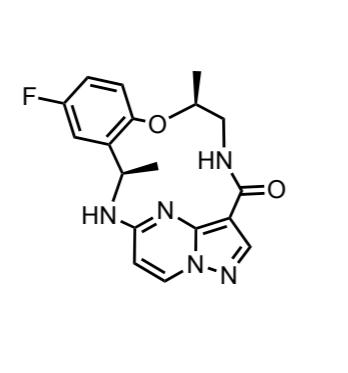
**motixafortide**  
(Aphexda)  
**CXCR4 inhibitor**

for stem cell mobilization in multiple myeloma  
1.25 mg/kg SQ  
**KYOTO UNIVERSITY (BIOLINERX)**



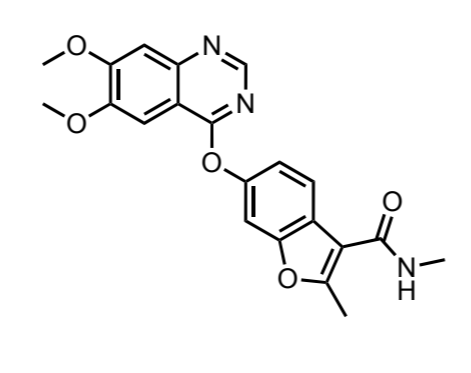
**nirogacestat**  
(Ogsiveo)  
**γ-secretase inhibitor**

for progressing desmoid tumors  
150 mg orally BID  
**PFIZER (SPRINGWORKS)**



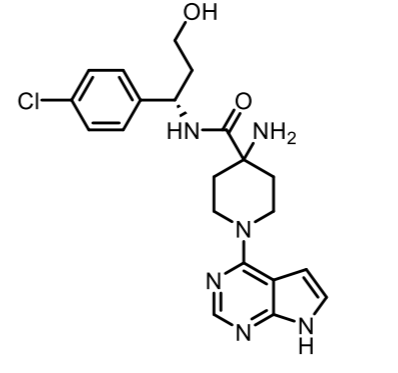
**repotrectinib**  
(Augtyro)  
**ROS1, TRKA/B/C inhibitor**

for ROS1+ non-small cell lung cancer  
160 mg orally QD then BID  
**TURNING POINT (BMS)**



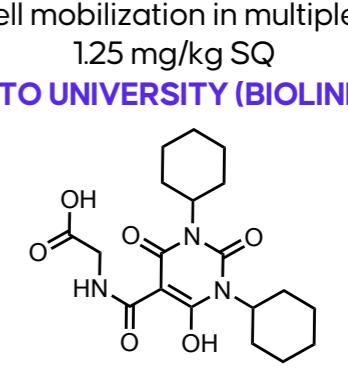
**fruquintinib**  
(Fruzaqla)  
**VEGFR1/2/3 inhibitor**

for refractory, metastatic colorectal cancer  
5 mg orally QD  
**HUTCHISON MEDIPHARMA (TAKEDA)**



**capivasertib**  
(Truqap)  
**AKT1/2/3 inhibitor**

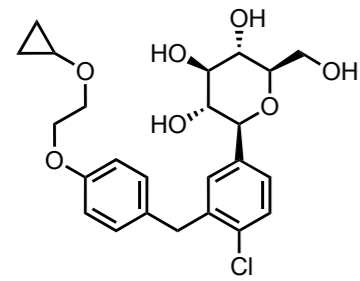
for HR+/HER2- breast cancer  
400 mg orally BID  
**ICR (ASTRAZENECA)**



**daproductat**  
(Jesduvroq)  
**HIF-PH1/2/3 inhibitor**

for anemia in chronic kidney disease  
1, 2 or 4 mg orally QD  
**GSK**

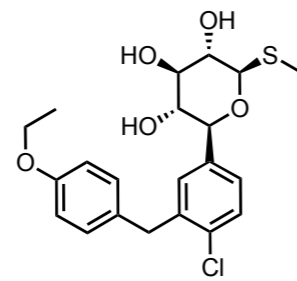
## Endocrinology (1)



**hexagliflozin**  
(Brenzavvy)  
**SGLT2 inhibitor**

for type 2 diabetes mellitus  
20 mg orally QD  
**THERACOSBIO**

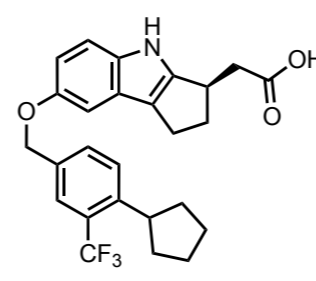
## Cardiology (1)



**sotagliflozin**  
(Inpefa)  
**dual SGLT1/2 inhibitor**

for reducing risk of CV death, HF hospitalization, and urgent HF visits  
200 or 400 mg orally QD  
**LEXICON**

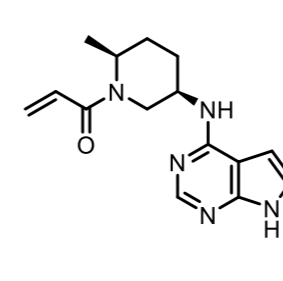
## Gastroenterology (1)



**etrasimod**  
(Velsipity)  
**S1P receptor modulator**

for ulcerative colitis  
2 mg orally QD  
**ARENA (PFIZER)**

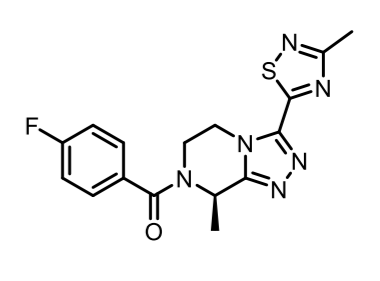
## Dermatology (1)



**ritlecitinib**  
(Litfulo)  
**JAK3/TEC kinase inhibitor**

for severe alopecia areata  
50 mg orally QD  
**PFIZER**

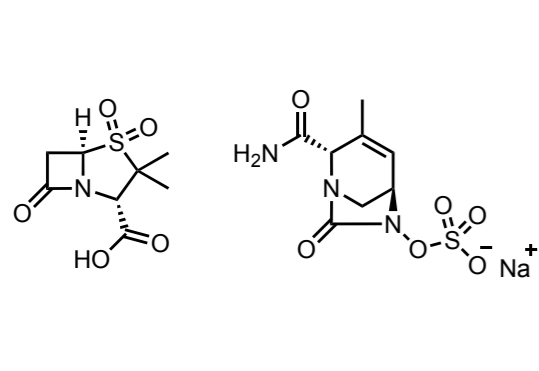
## OB/GYN (1)



**fezolinetant**  
(Veoza)  
**NK<sub>3</sub> receptor antagonist**

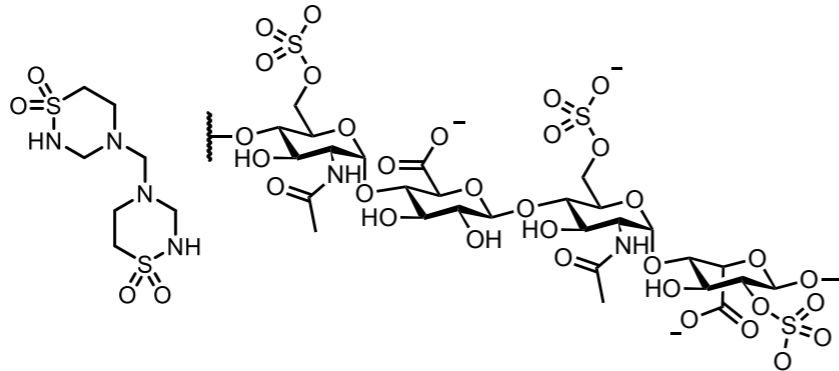
for hot flashes in menopause  
45 mg orally QD  
**EUROSCREEN (ASTELLAS)**

## Infectious Diseases (4)



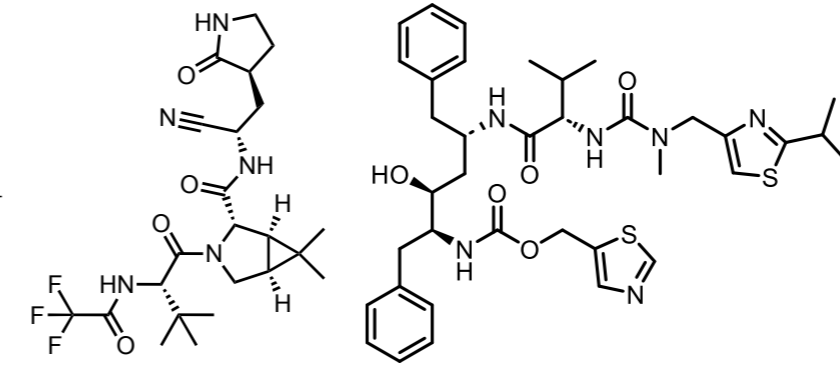
**sulbactam, durlobactam**  
(Xacduro)  
**β-lactamase inhibitors**

for HABP/VABP caused by *Acinetobacter baumannii-calcoaceticus*  
1 g of sulbactam, 1 g of durlobactam Q6H IV  
**ASTRAZENECA (ENTASIS)**



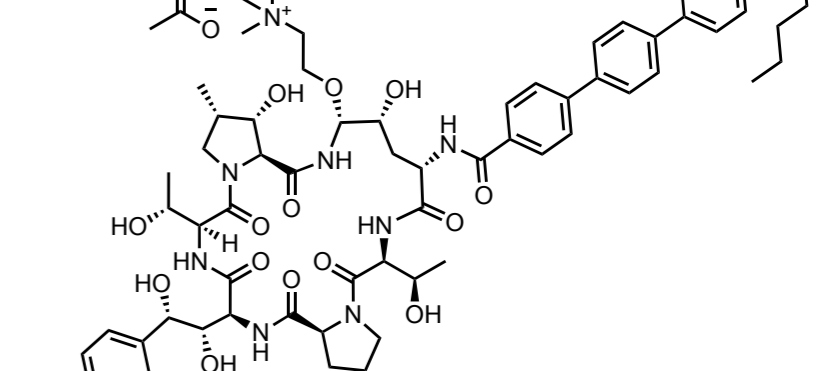
**taurolidine, heparin**  
(Defencath)\*\*\*  
**taurolidine: damages microbial cell walls**

**heparin: antithrombin III activator**  
for reducing incidence of CRBSI  
3 or 5 mL via CVC  
**CORMEDIX**



**nirmatrelvir, ritonavir**  
(Paxlovid)  
**nirmatrelvir: M<sup>Pro</sup> inhibitor;**

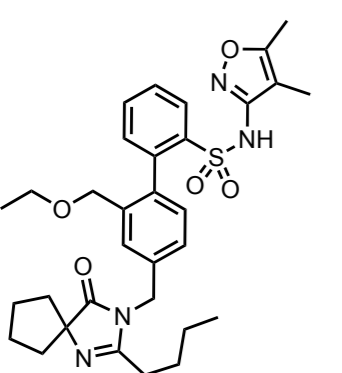
**ritonavir: CYP3A inhibitor**  
for COVID-19  
300 mg (nirmatrelvir) and 100 mg (ritonavir) orally BID  
**PFIZER**



**rezafungin**  
(Rezzayo)  
**1,3-β-D-glucan synthase inhibitor**

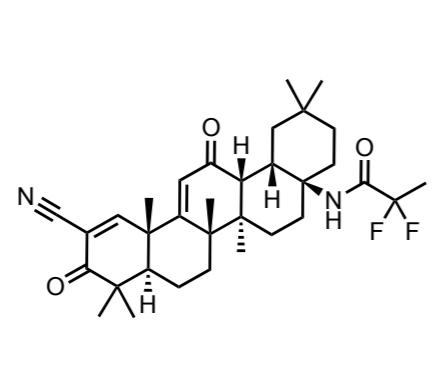
for candidemia and invasive candidiasis  
400 mg IV (loading dose) and then 200 mg IV QW  
**SEACHAID (MELINTA)**

## Rare Diseases (9)



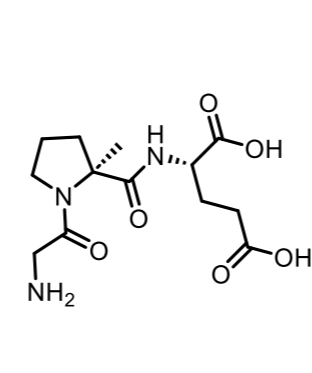
**sparsentan**  
(Filspari)\*  
**ET<sub>A</sub>R and AT<sub>1</sub>R antagonist**

for reducing proteinuria in IgAN  
400 mg orally QD  
**BMS (TRAVERE)**



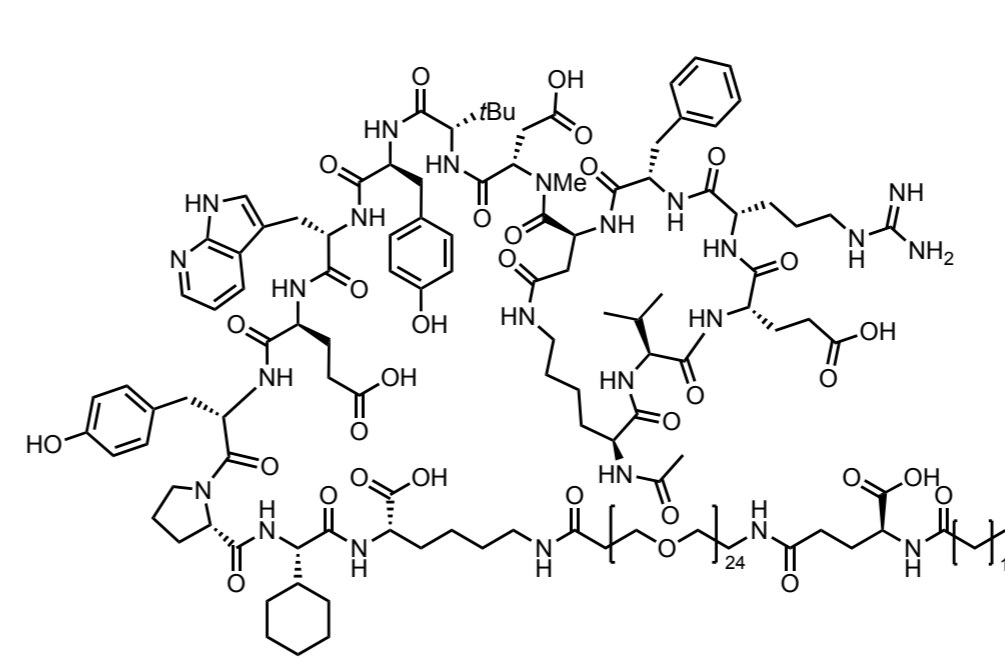
**omaveloxolone**  
(Skyclarys)  
**Nrf2 activator**

for Friedreich's ataxia  
150 mg orally QD  
**REATA**



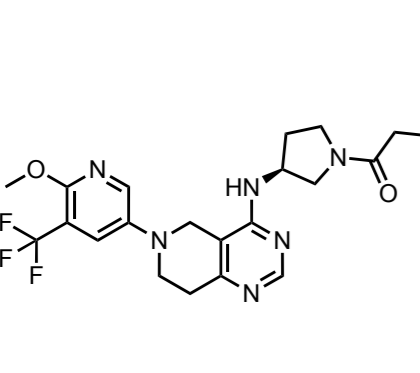
**trofinetide**  
(Daybue)  
**GPE tripeptide**

for Rett syndrome  
5, 6, 8, 10 or 12 g orally BID  
**NEUREN (ACADIA)**



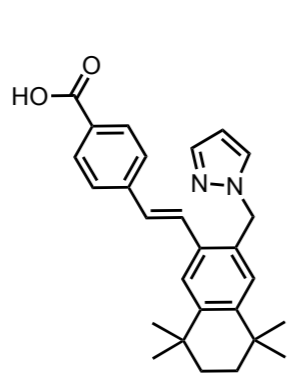
**zilucoplan**  
(Zilbrysq)  
**binds to C5 and inhibits its cleavage**

for myasthenia gravis  
16.6, 23 or 32.4 mg SQ  
**RA PHARMACEUTICALS (UCB)**



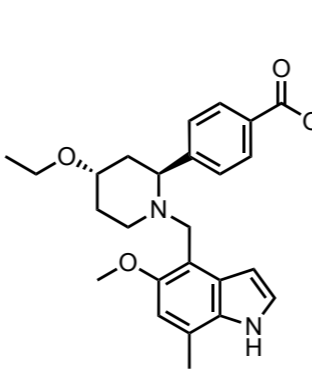
**leniolisib**  
(Joenja)  
**PI3Kδ inhibitor**

for activated phosphoinositide 3-kinase delta syndrome (APDS)  
70 mg orally BID  
**NOVARTIS (PHARMING)**



**palovarotene**  
(Sohonos)  
**RARγ agonist**

for fibrodysplasia ossificans progressiva  
5 mg orally QD  
**SYNTEX/ROCHE (IPSEN)**



**iptacopan**  
(Fabhalta)  
**complement factor B inhibitor**

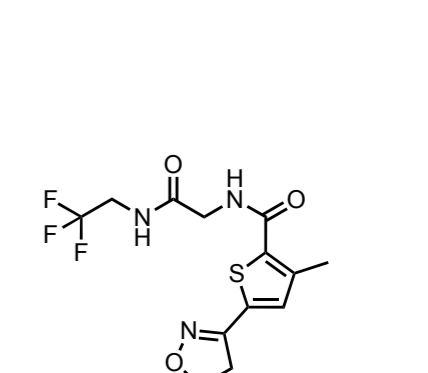
for paroxysmal nocturnal hemoglobinuria  
200 mg orally BID  
**NOVARTIS**

**vamorolone**  
(Agamree)  
**glucocorticoid receptor agonist**

for Duchenne muscular dystrophy  
6 mg/kg orally QD  
**REVERAGEN (SANTHERA)**

**perfluorohexyloctane\*\***  
(Miebo)  
**reduces evaporation**

for dry eye disease  
one drop QID  
**NOVALIQ (BAUSCH + LOMB)**

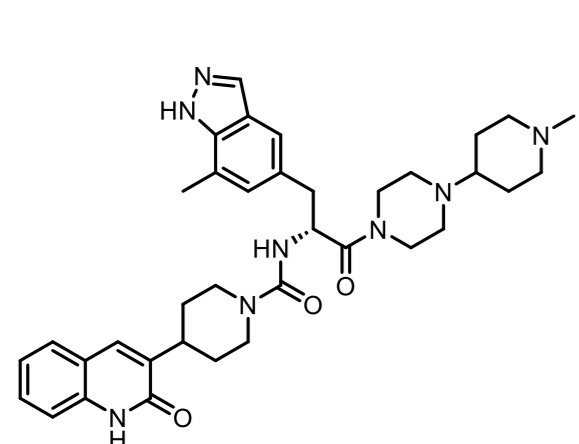


**lotilaner**  
(Xdemyv)  
**mite GABA-gated Cl<sup>-</sup> channel inhibitor**

for Demodex blepharitis  
one drop BID  
**NOVARTIS (TARSUS)**

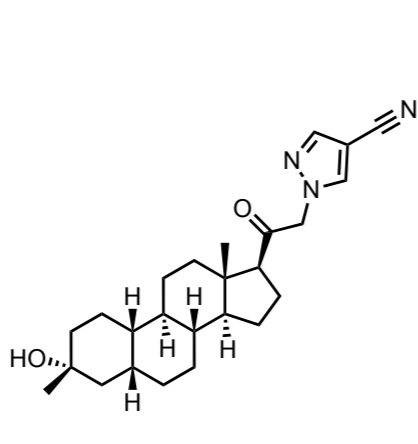
**birch triterpenes (Filsuvez) modulates inflammation, keratinocytes, and wound healing,**  
for wounds associated with dystrophic and junctional epidermolysis bullosa, 10% topical gel QD  
**AMRYT PHARMA (CHIESI GLOBAL RARE DISEASES)**

## Neurology (3)



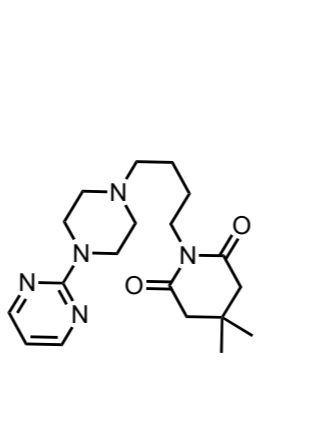
**zavegepant**  
(Zavzpret)  
**CGRP receptor antagonist**

for migraine  
10 mg nasally QD  
**BMS (PFIZER)**



**zuranolone**  
(GABA<sub>A</sub> PAM)  
**GABA<sub>A</sub> PAM**

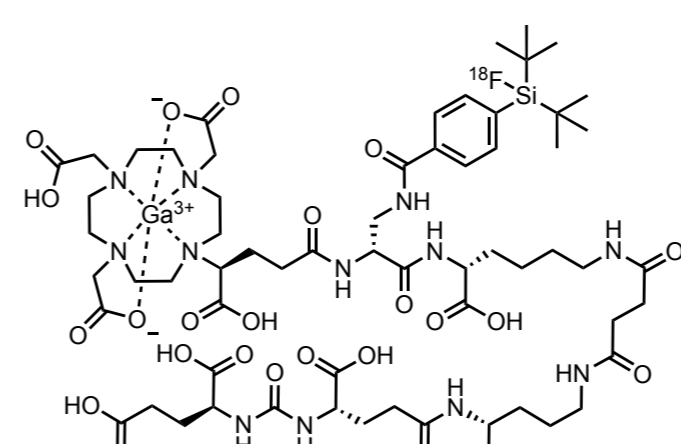
for postpartum depression  
50 mg orally QD  
**SAGE THERAPEUTICS**



**gepirone**  
(Exxua)  
**selective 5HT<sub>1A</sub> agonist**

for major depressive disorder  
18.2 to 72.6 mg orally QD  
**BMS (FABRE-KRAMER)**

## Diagnostic (1)



**flotufolstat F-18**  
(Posluma)  
**binds to PSMA expressed on cells**

for PET imaging of prostate cancer  
296 MBq IV  
**TU MUNICH (BLUE EARTH DIAGNOSTICS)**

## Footnotes

\* accelerated approval

\*\* perfluorohexyloctane was originally tested for anesthesia: WO1998040084A1

\*\*\* taurolidine and heparin were characterized in 1964 (GB1124285A) and 1916 (McLean, J. The discovery of heparin. *Circulation*, 1959, 19 (1), 75-78), respectively. The combination solution is novel.

Abbreviations:  
CV: cardiovascular, HF: heart failure, HABP: hospital-acquired bacterial pneumonia, VABP: ventilator-associated bacterial pneumonia, CVC: central venous catheter, CRBSI: catheter-related bloodstream infection, M<sup>Pro</sup>: SARS-CoV-2 main protease, IgAN: IgA nephropathy, GPE: glycine-proline-glutamic acid