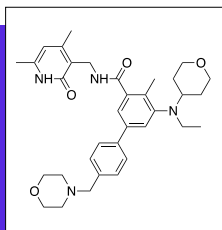
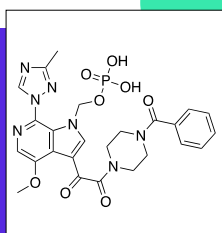
**ozanimod (Zeposia)**

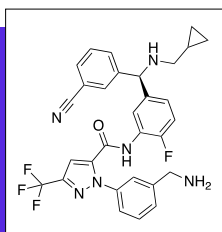
oral S1P(1/5) receptor modulator
0.92 mg QD
annualized relapse rate (ARR) vs. IFN β -1a
NCT02294058, 02047734 (895, 874 pts)
Neurology – multiple sclerosis

**tazemetostat (Tazverik)**

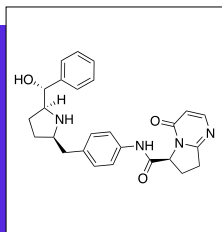
oral EZH2 methyltransferase inhibitor
800 mg BID
overall response rate (ORR)
Ph. II NCT02601950/EZH-202 (62 pts)
Oncology – epithelioid sarcoma

**fostemsavir (Rukobia)**

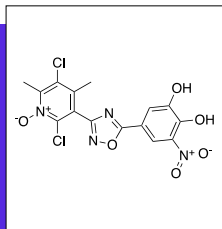
oral HIV-1 gp120 attachment inhibitor
600 mg BID
decline in HIV-1 RNA from d1-8 vs. placebo
NCT02362503/BRIGHT (371 pts)
Infectious Disease – HIV

**berotralstat (Orladeyo)**

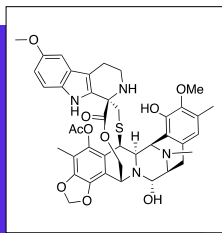
oral plasma kallikrein inhibitor
150 mg QD
HAE attacks vs. baseline attack rate
NCT3485911 (120 pts)
Hematology – hereditary angioedema

**vibegron (Gemtesa)**

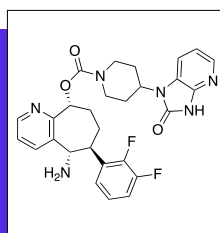
oral selective β_3 adrenergic receptor agonist
75 mg QD
avg. daily micturitions, UII ep. vs. baseline
NCT03492281/EMPOWUR (1515 pts)
Urology – Overactive bladder

**opicapone (Ongentys)**

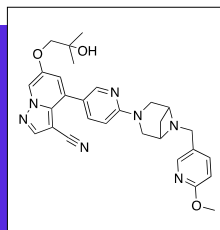
oral COMT enzyme inhibitor
50 mg QD at night
change in OFF-time based on pt. diaries
NCT01568073, 01227655 (600, 427 pts)
Neurology – Parkinson's w/ "off" episodes

**lurbinectedin (Zepzelca)**

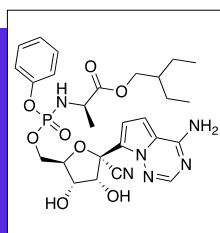
IV DNA alkylating agent
3.2 mg/m² Q21D over 60 minutes
overall response rate (ORR)
Ph. II NCT02454972 (105 pts)
Oncology – met. SCLC

**rimegepant (Nurtec ODT)**

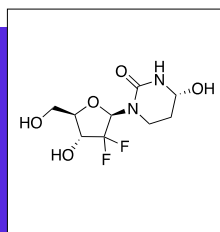
oral CGRP GPCR antagonist
75 mg
pain and MBS freedom vs. placebo
NCT03461757 (1466 pts)
Neurology – migraine

**selpercatinib (Retevmo)**

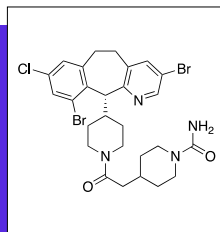
oral RET fusion kinase inhibitor (WT+mut)
160 mg BID (>50 kg)
overall response rate, duration of response
Ph. I/II NCT03157128/LIBRETTO-001 (304 pts)
Oncology – NSCLC, adv. RET+ MTC

**remdesivir (Veklury)**

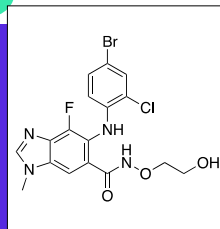
IV nucleotide RNA polymerase inhibitor
200 mg loading dose, 100 mg QD
time to recov., d11/14 clin. status category
04280705, 04292899/2730 (1062, 397, 584)
Infectious Disease – COVID-19

**cedazuridine (Inqovi)**

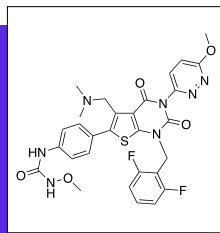
oral nucleoside cytidine deaminase inhibitor
100 mg QD (combo), d1-5 on 28-d cycle
CR %, transfus. dependence conversion rate
NCT02103478, NCT03306264 (80, 133 pts)
Oncology – myelodysplastic syndromes

**lonafarnib (Zokinvy)**

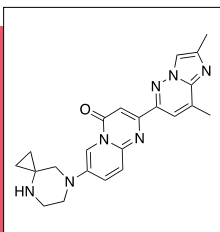
oral farnesyltransferase inhibitor
115 mg/m² BID, 150 mg/m² after 4 mo.
% pts w/ successful rate of weight gain
Ph. II 00425607, 00916747 (28, 26 pts)
Genetic Disorders – progeroid laminopathies

**selumetinib (Koselgo)**

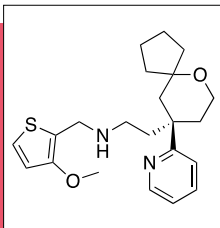
oral MEK1/2 kinase inhibitor
25 mg/m² BID
overall response rate (ORR)
Ph. II NCT01362803/SPRINT (50 pts)
Neuro-oncology – neurofibromatosis type 1

**relugolix (Orgovyx)**

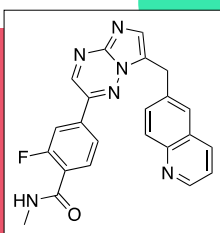
oral GnRH hormone receptor antagonist
360 mg d1, then 120 mg QD
medical castration rate/serum testost. levels
NCT03085095/HERO (934 pts)
Oncology – advanced prostate cancer

**risdiplam (Evrysdi)**

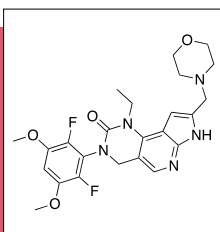
oral SMN RNA splicing modifier
5 mg (2 yrs of age + >20 kg)
ability to sit w/o support for at least 5 s
Ph. II/III 02913482, 02908685 (62, 231 pts)
Neurology – spinal muscular atrophy (SMA)

**oliceridine (Olinvyk)**

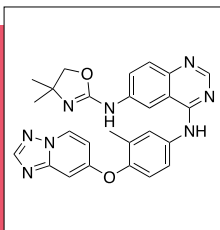
IV opioid receptor agonist
1.5 mg initially
pt. – reported pain after surgical procedures
Ph. II NCT02820324 (401 pts)
Neurology – acute pain in certain adults

**capmatinib (Tabrecta)**

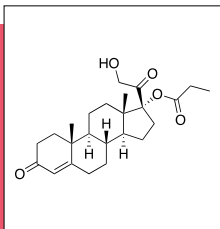
oral MET kinase inhibitor
400 mg BID
overall response rate (ORR)
Ph. II NCT02414139/GEOMETRY (97 pts)
Oncology – NSCLC

**pemigatinib (Pemazyre)**

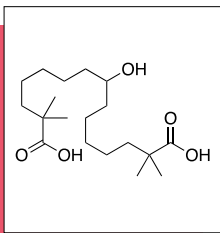
oral FGFR1/2/3 kinase inhibitor
13.5 mg QD for 14d, 7d off on 21d cycles
overall response rate, duration of response
Ph. II NCT02924376/FIGHT-202 (107 pts)
Oncology – adv. or met. Cholangiocarcinoma

**tucatinib (Tukysa)**

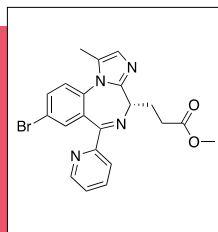
oral HER2 kinase inhibitor
300 mg BID
progression-free survival (PFS)
Ph. II NCT02614794/HER2CLIMB (612 pts)
Oncology – adv. unresect. or met. HER2+ BC

**clascoterone (Winlevi)**

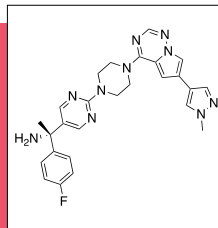
topical androgen receptor inhibitor
c.a. 1 g BID
% pts w/ IGA score change vs. baseline
NCT02608450, NCT02608476 (1421 pts)
Dermatology – acne vulgaris

**bempedoic acid (Nexletol)**

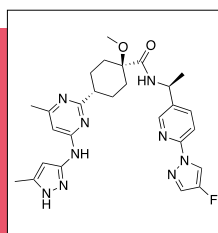
oral ATP citrate synthase enzyme inhibitor
180 mg QD
% change in LDL-C (wk 12) vs. baseline
NCT02666664, NCT02991118 (2230, 779 pts)
Cardiology – familial hypercholesterolemia

**remimazolam (Byfavo)**

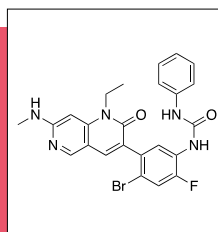
IV GABAA receptor modulator
5 mg over 1 min, supp. 2.5 mg over 15s
colonoscopy procedure success rate
02290873/6892, 02532647 (461, 431, 77 pts)
Neurology – sedation

**avapritinib (Ayvakit)**

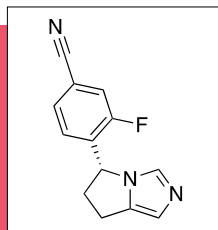
oral PDGFRα + KIT kinase inhibitor
300 mg QD
overall response rate (ORR)
Ph. I NCT02508532/NAVIGATOR (43 pts)
Oncology – unresectable or met. GIST

**pralsetinib (Gavreto)**

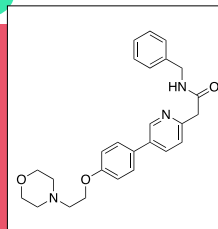
oral RET fusion kinase inhibitor (WT+mut)
400 mg QD
overall response rate, duration of response
Ph. I/II NCT03037385/ARROW (114 pts)
Oncology – NSCLC

**ripretinib (Qinlock)**

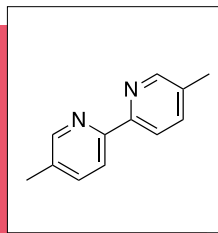
oral KIT + PDGFRα kinase inhibitor
150 mg QD
progression-free survival (PFS)
NCT03353753/INVICTUS (129 pts)
Oncology – adv. GIST

**osilodrostat (Isturisa)**

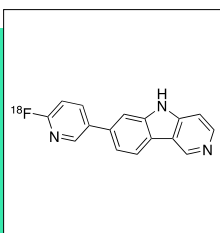
oral cortisol synthesis inhibitor
2 mg BID, titrated up to 30 mg BID max
compl. responder rate diff. after withdrawal
NCT02180217/LINC-3 (137 pts)
Endocrinology – Cushing's disease

**tirbanibulin (Klisyri)**

topical microtubule inhibitor
2.5 mg QD for 5d
clearance of AK lesions in treatment area
NCT03285477, NCT03285490 (702 pts)
Dermatology – actinic keratosis of face/scalp

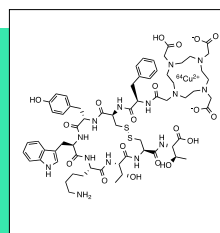
**abametapir (Xeglyze)**

topical metalloproteinase inhibitor
amount sufficient to coat hair and scalp
live lice freedom rate on days 1, 7, 14
Ph. I/II 02060903, 02062060 (379, 325 pts)
Infectious Disease – head lice



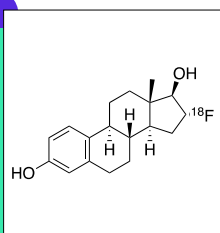
flortaucipir F18 (Tauvid)

IV tau protein aggregate radiodiagnostic
370 MBq
diagnostic performance of blinded readers
NCT02516046, NCT03901092 (156, 242 pts)
Neurology – Alzheimer's disease diagnostic



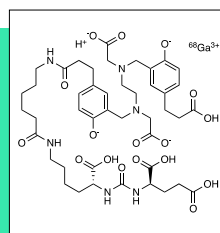
⁶⁴Cu dotatate (Detectnet)

IV somatostatin rcpt. (SSTR2) radiodiagnostic
148 MBq
blinded readers image interpret. agreement
NCT04334837
Oncology – SSTR+ NET diagnostic



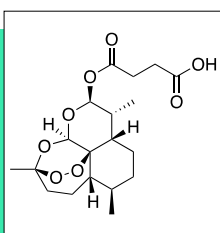
fluoroestradiol ¹⁸F (Cerianna)

IV ER-binding radiodiagnostic
222 MBq IV over 1–2 min
lesion-level reader agreement w/ biopsy
NCT01986569 (90 pts)
Oncology – breast cancer imaging



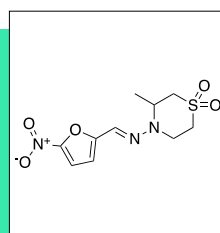
⁶⁸Ga PSMA-11

IV PSMA-binding radiodiagnostic
111 MBq
PET scan predictive value vs. histopath.
Ph. II/III 02919111, 02918357 (325, 635 pts)
Oncology – prostate cancer diagnostic



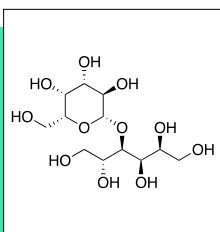
artesunate (Artesunate)

IV artemisinin-based antimalarial
2.4 mg/kg at 0 h, 12 h, 24 h
in-hospital mortality rate
international trials (Asia, Africa) (832 pts)
Infectious Disease – severe malaria



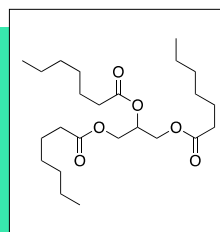
nifurtimox (Lampit)

oral antiprotozoal
8–10 mg/kg (>40 kg)
% pts serologically cured
NCT02625974 (330 pts)
Infectious Disease – Chagas disease



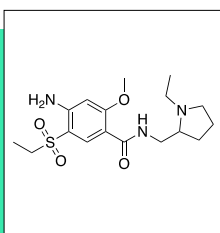
lactitol (Pizensy)

oral osmotic laxative
20 g QD
% pts w/ weekly responses for >9/12 wks
NCT02819297, NCT02481947 (594 pts)
Gastroenterology – constipation (CIC)



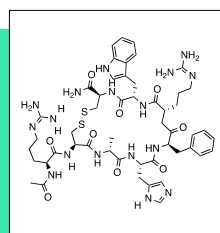
triheptanoin (Dojolvi)

oral fatty acid replacement
up to 35% of prescribed DCI over 4 doses
comparison with trioctanoin SOC
(32 pts)
Genetic Disorders – LC-FAOD



amisulpride (Barhemsy)

IV selective dopamine D2/3 receptor antag.
10 mg over 1–2 min
absence of emesis/rescue med. 24 h postop.
NCT01991860, NCT02337062 (342, 1147 pts)
Gastroenterology – postop. naus. + vomiting



setmelanotide (Imcivree)

subcutaneous MC4 receptor agonist
2 mg QD for 2 wks (>12 yrs old)
% change in body wt. from baseline (1 yr)
NCT02896192, NCT03287960 (21 pts)
Endocrinology – chron. weight management