Drug Pipeline 2026-2027

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Statement of Disclosure

- The speakers have no conflicts of interest with ineligible companies to disclose

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Learning Objectives

- Outline the FDA drug approval pathway, including expedited review programs and PDUFA target action dates.
- Describe recent FDA changes in clinical trial design, including single pivotal trial and real-time clinical trials.
- Identify emerging therapies with anticipated FDA approval over the next 12 months and discuss their novel mechanisms of action.
- Review efficacy, safety, and therapeutic niche of selected pipeline agents expected to impact acute care, specialty, and infusion pharmacy services.

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FDA Approval pathway

Lolic M. Food & Drug Administration (FDA). NDA at the FDA. Accessed August 14, 2026.

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FDA Drug Development Designations

Development

Fast Track
- Treats a serious condition and fills an unmet need
- Frequent FDA meetings; eligible for accelerated and priority review

Breakthrough Therapy
- A serious condition and preliminary clinical evidence of ***substantial*** improvement
- All fast track features plus intensive guidance and organizational commitment

Review

Priority Review
- Directs FDA attention and resources to drugs offering significant improvement
- Action within 6 months versus 10 months for standard review

Approval

Accelerated Approval
- Surrogate endpoints reasonably likely to predict benefit can support approval
- Confirmatory phase 4 trials required after approval

A single drug may carry more than one designation.

US Food & Drug Administration. Development & Approval Process (Drugs). Accessed August 12, 2026.

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Commissioner's National Priority Voucher **Pilot** Program

CNPV Program at a glance
- Ultra-accelerated priority review using a collaborative, tumor board-style review process
- Prioritizes review of products that align with strategic national importance

Presubmission requirements
- Sponsors engage the FDA and submit key materials before filing
- Front-loading the package makes the compressed timeline possible

Review in 1 to 2 months
- Target 1 to 2 months vs 6 months
- A multidisciplinary team reviews together rather than sequentially

National priority alignment
- Public health crises
- Breakthrough innovation
- Unmet need
- Onshoring of manufacturing
- Affordability of the product

US Food & Drug Administration. Commissioner's National Priority Voucher (CNPV) Pilot Program. Accessed August 12, 2026.

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Prescription Drug User Fee Act (PDUFA)

1992 PDUFA enacted to collect user fees on applications

The problem Concerns about timely review of applications

Before PDUFA Decisions took 21 to 29 months

Today 10 months for standard review

The "PDUFA date"
The goal date by which the FDA aims to act on an application, set when it is filed

Sibbesen JB, Rich J. Understanding the FDA Approval Process and PDUFA Dates.. Accessed August 12, 2026.

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Knowledge Check 1

A drug for a serious condition is approved on a surrogate endpoint reasonably likely to predict clinical benefit, with a confirmatory trial required after approval. Which program best fits with this definition?

A. Accelerated Approval
B. Priority Review
C. Fast Track Designation
D. Breakthrough Therapy Designation

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Recent FDA Updates – Single Pivotal Trial

- Historically 2 positive pivotal trials were expected to establish substantial evidence
- 2026 Approach: one trial plus confirmatory evidence

One adequate, well-controlled trial + **Confirmatory evidence** = **Same substantial evidence standard**

The evidentiary standard is unchanged — the path to meeting it is more flexible

Prasad V, Makary MA. N Engl Med. 2026

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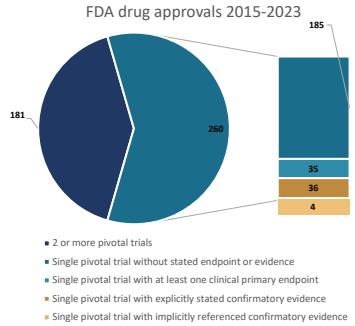
Prior to Single Pivotal Trial Model

>50% of drug approvals rested on a single pivotal trial



59%
Single pivotal trial
260 of 441 approvals

41%
Two or more pivotal trials
181 of 441 approvals

9%
Explicit confirmatory evidence cited
36 of 441 approvals

Godoy et al. Clinical Trials. 2025

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Real-Time Clinical Trials (RCT)

FDA initiative: predefined safety and efficacy data reach the agency while the trial is running

STANDARD TRIAL	REAL-TIME TRIAL
▶ Sites collect data throughout the study, held internally	▶ AI-enabled feeds transmit agreed upon endpoint signals
▶ Results packaged at set intervals	▶ FDA continuously reviews safety and efficacy
▶ FDA sees the evidence in one batch at submission	▶ Issues surface early, not at submission
▶ Review clock starts once the full package arrives	▶ Potential for faster decision-making

Two proof-of-concept trials are now underway to test the real-time model

Logan M. BMJ. 2026.

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Knowledge Check 2

Under the FDA's single pivotal trial approach, which statement correctly describes what is required to establish substantial evidence of effectiveness?

A. One adequate and well-controlled trial alone, with the evidentiary standard lowered
B. One trial with a surrogate endpoint, with no post-approval obligations
C. One adequate and well-controlled trial plus confirmatory evidence, meeting the same substantial evidence standard
D. Two adequate and well-controlled trials, as has always been required

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Symbols

- Oral therapy
- Injectable
- Infusion
- Topical
- Cell or Gene Therapy
- Ophthalmic
- Specialty Pharmacy
- Hospital or Clinic Administered
- Self-administered

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Select 2026 + 2027 Pipeline Agents

Anitocabtagene Autoleucel	Asundexian	Bepirovirsen	Bictegravir; lenacapavir	Cagrilintide/ semaglutide
Denecimig	Giredestrant	Ianalumab	Ifinatamab	Imsidolimab
Insulin Efsitora Alfa	Lirafugratinib	Neladalkib	Ozekibart	Pelacarsen
Povorcitinib	Pritelivir	Rebisufligene Etisparvovec	Varegacestat	Zipalertinib

Brand and Biosimilar Pipeline Database [proprietary data]. IPD Analytics; 2026.

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Lirafugratinib

Anticipated Place in Therapy
- Second line option for pretreated cholangiocarcinoma with FGFR2 fusions or rearrangements
- First highly selective, irreversible FGFR2 inhibitor
- FDA PDUFA date = September 27, 2026

Important Trials
- ReFOCUS Cohort: patients with FGFR inhibitor-naïve CCA
- ORR 46.5% (95% CI, 37.1%-56.1%)

Mechanism of Action
- FGFR2 tyrosine kinase inhibitor - blocks FGFR2 signaling pathway

Dosing and Administration
- 70 mg once daily orally

Hollebecque A. J Clin Oncol 44, 2026

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Giredestrant

Anticipated Place in Therapy
- New generation selective estrogen receptor degrader (SERD)
- Significant advancement in adjuvant endocrine therapy in ~20 years
- FDA PDUFA Date = November 30, 2026

Important Trials
- Phase III evERA Breast Cancer study: everolimus with giredestrant or SOC endocrine therapy
- Reduced risk of invasive disease recurrence or death by 30% vs SOC endocrine therapy/everolimus (HR=0.70, 95%CI 0.57-0.87).

Mechanism of Action
- Blocks oestrogen from binding to the receptor triggering the breakdown (degradation) and stopping/slowing growth

Dosing and Administration
- 30 mg orally once daily

Joseph R, et al. Am J Health Syst Pharm. 2026.
Roche. FDA accepts New Drug Application for Roche's giredestrant in ER-positive early-stage breast cancer, the first and only oral SERD with positive phase III results in the curative setting. Accessed August 12, 2026.

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Ifinatamab deruxtecan

Anticipated Place in Therapy
- Adult patients with extensive-stage small cell lung cancer with disease progression or after platinum-based chemotherapy
- FDA PDUFA Date = October 10, 2026

Important Trials
- Phase 2 IDeate-Lung01 Trial
- ORR 48.2% - median duration 5.3 months
- 9-month overall survival ~59.1%

Mechanism of Action
- First-in-class B7-H3 directed antibody drug conjugate

Dosing and Administration
- 12 mg/kg IV every 3 weeks

Merck. Ifinatamab Deruxtecan Granted Breakthrough Therapy Designation by U.S. FDA for Patients with Pretreated Extensive-Stage Small Cell Lung Cancer. Accessed August 12, 2026.
1. Martin C. International Association for the Study of Lung Cancer. Accessed August 12, 2026.

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Neladalkib

Anticipated Place in Therapy
- NSCLC with diverse ALK fusions and resistance mutations
- A "4th generation" ALK inhibitor for previously treated disease
- FDA PDUFA date = November 27, 2026

Important Trials
- ALKOVE-1 (phase I/II) in advanced ALK-positive NSCLC
- ORR 31% after prior ALK TKI therapy; ORR 86% in TKI-naive patients

Mechanism of Action
- Brain-penetrant, ALK-selective tyrosine kinase inhibitor
- Designed to cover diverse ALK fusions and resistance mutations

Dosing and Administration
- 150 mg daily orally

Lin JJ et al. J Clin Oncol. 2026
Doherty K. OncLive. FDA Grants Priority Review to Neladalkib for TKI-Pretreated ALK+ NSCLC. Accessed August 12, 2026.

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Zipalertinib

Anticipated Place in Therapy
- Locally advanced or metastatic NSCLC with EGFR exon 20 insertion mutations
- Post-platinum, with or without prior amivantamab; an oral option where targeted choices are limited
- FDA PDUFA date = February 27, 2027

Important Trials
- REZILIENT1 (phase 2b): zipalertinib monotherapy after prior systemic therapy; primary endpoint ORR
- Confirmed ORR 35% (n=176), median DOR 8.8 months; ORR 40% after platinum only (n=125)

Mechanism of Action
- Oral, next-generation irreversible EGFR tyrosine kinase inhibitor
- Targets exon 20 insertion and other activating EGFR variants while sparing wild-type EGFR

Dosing and Administration
- 100 mg orally twice daily

Doherty K, Mackay K. OncLive.FDA Accepts NDA for Zipalertinib in Locally Advanced or Metastatic EGFR Exon 20+ NSCLC. Accessed August 12, 2026.

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Ozekibart

Anticipated Place in Therapy
- Adults with unresectable or metastatic grade 2 or 3 conventional chondrosarcoma
- Would be first and only FDA approved treatment
- FDA PDUFA date = April 14, 2027

Important Trials
- ConDRAgon (phase 3) in inoperable or metastatic conventional chondrosarcoma
- Improved PFS: 5.52 months vs 2.66 months (HR 0.479; 95% CI 0.33-0.68; p<0.0001)

Mechanism of Action
- Precision-engineered tetravalent death receptor 5 (DR5) agonist antibody
- Clusters and activates DR5 to trigger tumor cell apoptosis without requiring Fc crosslinking

Dosing and Administration
- 3 mg/kg IV every 3 weeks

Inhibrx. Inhibrx Announces U.S. FDA Acceptance of BLA for Ozekibart in Patients with Conventional Chondrosarcoma. Accessed August 12, 2026.

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Varegacestat

Anticipated Place in Therapy
- Adults with desmoid tumors
- An option for a rare soft tissue tumor with limited systemic therapies – potential to be SOC
- FDA PDUFA Date = April 28, 2027

Important Trials
- RINGSIDE trial in adults with progressing desmoid tumors
- PFS HR 0.16 (95% CI 0.071-0.375; p<0.0001), an 84% reduction in progression or death; confirmed ORR 56% vs 9%

Mechanism of Action
- Gamma-secretase inhibitor (GSI)
- Blocks notch signaling implicated in desmoid tumor growth

Dosing and Administration
- 1.2 mg once daily

Ryan C. OncLive. Varegacestat Represents Potential SOC for Progressing Desmoid Tumors. Accessed August 12, 2026.

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Novel Therapy Approvals?

Therapy	Indication
Daraxonrasib	Pancreatic cancer
Vusolimogene oderparepvec	Melanoma
Deramiocel	Cardiomyopathy associated with Duchenne muscular dystrophy
Pariglasgene brecaparvovec	Von Gierke disease (glycogen storage disease type I)

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Rebisufligene Etisparvovec

Anticipated Place in Therapy
- Treatment of Sanfilippo syndrome Type A (MPS IIIA)
- Potential first disease-modifying therapy
- FDA PDUFA Date = September 19, 2026

Important Trials
- Data reduced levels of CSF-HS (heparan sulfate) within first month
- Median reduction in CSF-HS was 63.98%

Mechanism of Action
- In vivo AAV9 gene therapy
- Delivers a healthy copy of the SGSH gene

Dosing and Administration
- One time IV infusion

Ultragenyx. Ultragenyx Announces Positive Longer-Term Data Demonstrating Treatment with UX111 Gene Therapy Results. Accessed August 12, 2026.

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Anitocabtagene Autoleucel

Anticipated Place in Therapy
- Multiple myeloma
- FDA PDUFA date December 23, 2026

Important Trials
- Phase 2 iMMagine-1 study
- 74% achieve stringent CR or CR
- OS rates 94% at 12 months, 88% at 18 months, 83% at 24 months

Mechanism of Action
- BCMA-directed CAR T-cell therapy
- D-domain technology → reduce toxicities

Dosing and Administration
- IV infusion – single dose in phase 2

Gilead. Kite Announces New Data for Pivotal iMMagine-1 Study at ASH 2025, Accessed August 12, 2026.

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Bepirovirsen

Anticipated Place in Therapy
- First line for chronic hepatitis B infection along with nucleotide analog (NA) therapy

FDA PDUFA date October 26, 2026

Important Trials
- B-WELL-1 and B-WELL-2
  - Bepirovirsen compared to placebo (in addition to SOC NA) showing functional cure in treatment arm in 19-20% of patients.

Mechanism of Action
- Antisense oligonucleotide that recruits liver enzymes to degrade hepatitis B RNA thereby reducing viral replication and production of HBV antigens

Dosing and Administration
- 300mg subcutaneously weekly for 6 months

Hou et al. NEJM. 2026

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Bictegravir; lenacapavir

Anticipated Place in Therapy
- HIV-1 treatment - single oral tablet regimen
- PDUFA date: August 27, 2026

Important Trials
- ARTISTRY-1 and ARTISTRY-2 trials
- Non-inferiority to other multi-tablet HIV-1 treatment regimens
- Less complex oral option for treatment-experienced and treatment-naïve patients

Mechanism of Action
- Bictegravir: integrase strand-transfer inhibitor (INSTI) with high resistance barrier
- Lenacapavir: capsid inhibitor (first in class)

Dosing and Administration
- Bictegravir 75 mg/lenacapavir 50 mg orally once daily

Orkin et al. The Lancet. 2026

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Pritelivir

Anticipated Place in Therapy
- Oral agent for treatment of HSV-1 in immunocompromised patient population, competing with currently available IV treatment options

Anticipated FDA approval late 2026

Important Trials
- PRIOH-1: Phase 3 trial comparing pritelivir to the standard of care (SOC) in immunocompromised patients ≥16 years with refractory HSV infection with or without resistance to acyclovir or foscarnet
  - Met primary endpoint of lesion healing at 28 days in 62.7% of pritelivir compared to 34% of placebo patients

Mechanism of Action
- DNA-helicase primase inhibitor

Dosing and Administration
- 400mg orally day 1, 100mg orally daily thereafter for 28 day course (in PRIOH-1 extended additional 14 days if necessary)

Papanicolaou et al. Transplantation and Cellular Therapy. 2026

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Povorcitinib

Anticipated Place in Therapy
- First oral targeted therapy for hidradenitis supparativa (HS) competing with other biologics for moderate to severe HS
- In clinical development for nonsegmental vitiligo, prurigo nodularis, and asthma

FDA approval anticipated in 1Q 2027

Important Trials
- STOP-HS1 and STOP-HS2
  - Phase 3 trials evaluating povorcitinib compared to placebo in adult patients with moderate to severe HS and inadequate response to ≥ oral antibiotic or biologic
  - Primary endpoint met of HS clinical response 50 (HiSCR50) compared to placebo at week 12 with increased response over time

Mechanism of Action
- Janus kinase (JAK) 1 Inhibitor

Dosing and Administration
- 45 mg or 75mg orally daily

Porter et al. Nature. 2026

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Ianalumab

Anticipated Place in Therapy
- Breakthrough therapy designation - first targeted treatment for Sjögren's disease (SjD)
- Along with eltrombopag, short-course second-line therapy in immune thrombocytopenia (ITP)
- Estimated FDA approval in second half of 2026

Important Trials
- NEPTUNUS-1 & NEPTUNUS-2: first replicate phase 3 studies showing statistically significant ESSDAI improvement vs placebo
- Ianalumab + eltrombopag in ITP: statistically significant increase in time to treatment failure vs placebo

Mechanism of Action
- Human IgG1 mAb that depletes B cells through enhanced antibody-dependent cellular cytotoxicity (ADCC)
- Also inhibits B-cell activation and survival via BAFF-R blockade

Dosing and Administration
- SjD: 300 mg SC monthly vs every 3 months (NEPTUNUS-2: no statistically significant difference between groups)
- ITP: 3 mg/kg vs 9 mg/kg IV monthly x 4 months

Beck et al. American College of Rheumatology Conference. 2025.
Cuker et al. NEJM. 2025

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Imsidolimab

Anticipated Place in Therapy
- Targeted treatment for generalized pustular psoriasis (GPP) flares in competition with spesolimab
- FDA PDUFA date: December 12, 2026

Important Trials
- GEMINI-1 and GEMINI-2
  - In GEMINI-1 one loading dose showed statistically significant improvement of GPP Physician Global Assessment (GPPPGA) score at week 4 compared to placebo
  - GEMINI-2 studied maintenance dosing showing patients on maintenance dosing remained with a GPPPGA score of 0/1 with no flares compared to placebo

Mechanism of Action
- Interleukin-36 receptor (IL-36R) antagonist

Dosing and Administration
- Loading dose: 750mg IVPB x 1
- Maintenance dose: 200mg subcutaneously q4 weeks

Smieszek et al. NEJM. 2026

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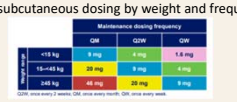
Denecimig

Anticipated Place in Therapy
- Bleeding prophylaxis in adult and pediatric patients with hemophilia A, with or without factor VIII inhibitors
- FDA approval expected in the second half of 2026

Important Trials
- FRONTIER program; FRONTIER-4
- Denecimig prophylaxis superior to on-demand treatment in preventing bleeding events in hemophilia A

Mechanism of Action
- Bispecific antibody mimicking activated factor VIII

Dosing and Administration
- Tiered subcutaneous dosing by weight and frequency



Mancuso et al. NEJM. 2026

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Asundexian

Anticipated Place in Therapy
- First in class Factor XIa inhibitor for secondary prevention of non-cardioembolic ischemic stroke or transient ischemic attack

FDA approval expected in November 2026

Important Trials
- OCEANIC-STROKE: Phase 3 trial in patients post non-cardioembolic ischemic stroke or high-risk TIA taking standard antiplatelet therapy, ischemic stroke was reduced by 26% compared to placebo

Mechanism of Action
- Coagulation factor XIa (FXIa) inhibitor

Dosing and Administration
- 50mg orally once daily in addition to planned single or double anti-platelet therapy

Sharma et al. NEJM. 2026

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Pelacarsen

Anticipated Place in Therapy

- First in class agent targeting elevated Lp(a) for cardiovascular risk reduction, offering a treatment option for secondary prevention in patients with established CVD/ASCVD and elevated Lp(a)
  - Other agents with similar mechanisms currently entering phase 3 trials

Anticipated FDA approval in 2027 with granted fast-track status

Important Trials

- Phase 2 dose finding study showed dose-dependent Lp(a) reductions of 35% to 80% compared to 6% with placebo – 20mg weekly having highest response
- Phase 3 Lp(a)HORIZON phase 3 trial ongoing

Mechanism of Action

- Apolipoprotein(a) (Apo[a]) mRNA translation inhibitor

Dosing and Administration

- Anticipated to be 80mg subcutaneously every month based on phase 2 dose finding study (current dosing in Lp(a) HORIZON trial)

Cho et al. American Heart Journal. 2025

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Insulin Efsitora Alfa

Anticipated Place in Therapy

- Basal insulin for type 1 and type 2 diabetes mellitus
- Estimated FDA approval in second half of 2026

Important Trials

- QWINT-1, QWINT-3 and QWINT-4 phase 3 clinical trials
- All three met the primary endpoint of non-inferior A1c reduction compared to daily basal insulin

Mechanism of Action

- Fusion protein: single-chain insulin analog linked to the fragment crystallizable (Fc) domain of human immunoglobulin G2
- Results in more controlled glucose uptake and sustained insulin activity

Dosing and Administration

- Variable subcutaneous dosing based on glucose control, once weekly
- QWINT-1 used a fixed-dose regimen in insulin-naïve patients with only four titration options

Tran. Journal of Clinical Diabetes. 2025

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Cagrilintide/semaglutide

Anticipated Place in Therapy

- Novel amylin analog + GLP-1 RA therapy for weight loss
- Will likely have to compete amongst available GLP-1/GIP agents

Important Trials

- REDEFINE – 1: Showed >20% weight loss in patients in cagrilintide/semaglutide arm compared to placebo over 68 weeks
- REDEFINE – 4: compared directly to tirzepatide, patients on tirzepatide saw weight loss of 23.6% compared to those on cagrilintide/semaglutide of 20.2%

Mechanism of Action

- Amylin analog and GLP-1 receptor agonist

Dosing and Administration

- Variable titrated subcutaneous dosing starting at 2.4mg cagrilintide /2.4mg semaglutide weekly

Garvey et al. NEJM. 2025

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Knowledge Check 3

Which pipeline agent is a first-in-class Factor XIa inhibitor for secondary prevention of non-cardioembolic ischemic stroke or TIA?

A. Asundexian
B. Pelacarsen
C. Denecimig
D. Ianalumab

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Knowledge Check 4

Which agent is a precision-engineered tetravalent death receptor 5 (DR5) agonist antibody designed to trigger apoptosis in tumor cells?

A. Varegacestat
B. Neladalkib
C. Ozekibart
D. Ifinatamab deruxtecan

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Summary

- Expedited review programs and the CNPV pilot are designed to shorten time to approval.
- New FDA programs (eg, single pivotal trial, real-time clinical trials) improve efficiency without changing the evidentiary standard.
- Emerging therapy approvals are expected over the next 12 months, many with first-in-class mechanisms.
- Anticipate formulary review, infusion capacity, and specialty onboarding needs for these niche therapies.

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