Compilation of CDER New Molecular Entity (NME) Drug and New Biologic Approvals – Data Dictionary

This dataset provides publicly available data on CDER NME and new biologic approvals (1985-2024) in a single file, analyzable, and user-friendly format. This dataset is provided for research purposes only, and some fields have been simplified for ease of presentation. This dataset is a high-level compilation of existing, publicly available data from FDA internal databases and document records, and to the best of our knowledge, reflects the state of each application at the time of initial regulatory approval. For additional or more detailed information about an application (e.g., FDA-approved conditions of use, approval letters), consider reviewing information available on Drugs@FDA (https://www.accessdata.fda.gov/scripts/cder/daf/index.cfm) or in the Orange Book (https://www.accessdata.fda.gov/scripts/cder/ob/index.cfm). The Agency aims to provide accurate information in this dataset and on this website. If you believe there is a factual error in the information presented, you can report this to CDER.NMENewBiologicApprovals@fda.hhs.gov.

New Molecular Entity (NME)

Definition: A New Molecular Entity (NME) is an active ingredient that contains no active moiety that has been previously approved by the Agency in an application submitted under section 505 of the Federal Food, Drug, and Cosmetic Act or has been previously marketed as a drug in the United States (see MAPP 5018.2 NDA Classification Codes).

New Biologic License Application (BLA)

Definition: Biological products, like other drugs, are used for the treatment, prevention, or cure of disease in humans. In contrast to chemically synthesized small molecular weight drugs, which have a well-defined structure and can be thoroughly characterized, biological products are generally derived from living material--human, animal, or microorganism-- are complex in structure, and thus are usually not fully characterized.

Section 351 of the Public Health Service (PHS) Act defines a biological product as a "virus, therapeutic serum, toxin, antitoxin, vaccine, blood, blood component or derivative, allergenic product, or analogous product, … applicable to the prevention, treatment, or cure of a disease or condition of human beings." FDA regulations and policies have established that biological products include blood-derived products, vaccines, in vivo diagnostic allergenic products, immunoglobulin products, products containing cells or microorganisms, and most protein products. Biological products subject to the PHS Act also meet the definition of drugs under the Federal Food, Drug and Cosmetic Act (FDC Act). Note that hormones such

as insulin, glucagon, and human growth hormone are regulated as drugs under the FDC Act, not biological products under the PHS Act. Monoclonal antibodies for in vivo use.

Per the Therapeutic Biologic Transfer Agreement enacted on June 30th, 2003. CDER has jurisdiction over the therapeutic biologic types listed below:

Proteins intended for therapeutic use, including cytokines (e.g., interferons), enzymes (e.g., thrombolytics), and other novel proteins, except for those that are specifically assigned to CBER (e.g., vaccines and blood products). This category includes therapeutic proteins derived from plants, animals, or microorganisms, and recombinant versions of these products.

Immunomodulators: proteins or peptides that are not antigen specific (e.g., cytokines, growth factors, chemokines, etc.) that are intended to treat disease by inhibiting or modifying a pre-existing immune response; and proteins or peptides intended to act in antigen-specific fashion to treat or prevent autoimmune diseases by inhibiting or modifying pre-existing immune responses.

Growth factors, cytokines, and monoclonal antibodies intended to mobilize, stimulate, decrease or otherwise alter the production of cells in vivo.1 This category includes growth factors, cytokines, and monoclonal antibodies, as well as non-biological agents, administered as mobilizing agents for their direct therapeutic effect on the recipient, as well as growth factors, cytokines, and monoclonal antibodies administered for the purpose of subsequently harvesting the mobilized, stimulated, decreased or otherwise altered cells for use in a human cellular or tissue-based product (HCT/P).

Note on Converted NDA to BLA Applications in Dataset:

A number of products listed in the dataset have been administratively changed from NDAs to BLAs. They are identified as such in the comments field. The reason for this change is cited below:

Beginning on March 23, 2020, the BPCI Act requires that an approved marketing application for a "biological product" under section 505 of the FD&C Act shall be deemed to be a license for the biological product (i.e., an approved BLA) under section 351 of the PHS Act. The 10-year transition period between the law's enactment on March 23, 2010, and March 23, 2020, provides sponsors of biological products that are affected by the statutory transition with time to prepare for the transition, and allows biological products submitted under section 505 of the FD&C Act time to be approved before March 23, 2020.

https://www.fda.gov/drugs/guidance-compliance-regulatory-information/deemed-be-license-provision-bpci-act

Abbreviations

BLA	Biologics License Application
BTD	Breakthrough Therapy Designation
CDER	Center for Drug Evaluation and Research
CFR	Code of Federal Regulations
FDA	Food and Drug Administration
FDAAA	Food and Drug Administration Amendments Act
FDASIA	Food and Drug Administration Safety and Innovation Act
FDAMA	Food and Drug Administration Modernization Act
FDCA	Food, Drug and Cosmetic Act
HHS	US Department of Health and Human Services
IND	Investigational New Drug Application
MAPP	Manual of Policies and Procedures
NDA	New Drug Application
NME	New Molecular Entity
OPD	Orphan Product Database
PDUFA	Prescription Drug User Fee Act
PHS	Public Health Service

Data Field Definitions

Abbreviated Indication(s)

Definition: The "abbreviated indication" for the purposes of this document, are derived from the approval letter issued to the applicant and/or INDICATIONS AND USAGE section of the labeling at the time of the original approval of the application. The abbreviated indication(s) are for research purposes only and may not reflect the full indication(s) statement for the products at the time of the original approval or the currently approved labeling. Supplemental indication(s) approved after the initial approval date are not listed at this time.

Multiple indications are included in the dataset only when such indications were approved simultaneously and at the time of the first approval for the NME. Otherwise, the dataset only includes the first approved indication for the product. In addition, letter designations (such as [A] and [B]) were used to delineate separate indications if such indications had different designations (e.g., Fast Track, Accelerated Approval).

The listed "abbreviated indication" on this website are for research purposes only. To see the FDA-approved conditions of use [e.g., indication(s), population(s), dosing regimen(s)] for each of these products, see the most recent FDA-approved Prescribing Information.

Accelerated Approval

Definition: A novel drug approval is considered Accelerated Approval if the drug that treats a serious condition, generally provides a meaningful advantage over available therapies, and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality (IMM) that is reasonably likely to predict an effect on IMM or other clinical benefit (i.e., an intermediate clinical endpoint). Marketing of the drug product and related activities must adhere to 21CFR 601.41 Subpart E for BLAs and 21CFR 314.510 Subpart H for NDAs.

Drugs under an Accelerated Approval indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

For drugs initially approved for multiple indications, both accelerated approval and regular approval, the subset of indication(s) for which accelerated approval was granted is specified.

For drugs approved prior to April 15, 1992 (the date notice was published to the Federal Register proposing the accelerated approval program), accelerated approval is listed as “N/A”.

Active Ingredient/Moiety

Definition: Active moiety is the molecule or ion, excluding those appended portions of the molecule that cause the drug to be an ester, salt (including a salt with hydrogen or coordination bonds), or other noncovalent derivative (such as a complex, chelate, or clathrate) of the molecule, responsible for the physiological or pharmacological action of the drug substance [see 21 CFR 314.3(b)]

Active ingredient is any component that is intended to furnish pharmacological activity or other direct effect in the diagnosis, cure, mitigation, treatment, or prevention of disease, or to affect the structure or any function of the body of man or other animals. The term includes those components that may undergo chemical change in the manufacture of the drug product and be present in the drug product in a modified form intended to furnish the specified activity or effect [see 21 CFR 314.3(b)].

Naming conventions follow Structured Patient Labeling.

Applicant

Definition: In common usage, the applicant is the primary agent who submits an application for marketing approval under section 505 of the Federal Food, Drug, and Cosmetic Act or section 351 of the Public Health Service Act. The name of the applicant is listed in the approval letter. The applicant name may be simplified in this database for presentation purposes.

Application Number(s)

Definition: The application number is a unique internal FDA tracking number assigned to an NDA or BLA upon its submission for review. One drug can have more than one application number if the drug has different dosage forms or more than one original indication. Multiple application numbers are listed in the same order the dosage form and route of administration appear.

Some BLAs are given new application numbers after the original approval due to administrative purposes. If a BLA is given a new application number, the new BLA number is listed in the “notes” column of the data set.

Approval Year

Definition: The approval year is the calendar year of the FDA Approval Date.

Approved Use(s)

Definition: The "approved use" is populated from the INDICATIONS AND USAGE section of the labeling (in the Full Prescribing Information) at the time of the original approval of the application. These detailed indication statements are provided for approvals occurring from October 1, 2007 to the stamped date of the dataset. Supplemental indication(s) approved after the initial approval date are not listed

Letter designations (such as [A] and [B]) were used to delineate separate indications if such indications had different designations (e.g., Fast Track, Accelerated Approval).

For more information about a drug's indication, see 21 CFR 201.57(c)(2) and the Draft Guidance for Industry: *Indications and Usage Section of Labeling for Human Prescription Drug and Biological Products – Content and Format* (July 2018).

The listed "approved uses" on this website are for research purposes only. To see the FDA-approved conditions of use [e.g., indication(s), population(s), dosing regimen(s)] for each of these products, see the most recent FDA-approved Prescribing Information.

Breakthrough Therapy Designation (BTD)

Definition: Breakthrough Therapy Designation may be granted to a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on one or more clinically significant endpoints. A novel drug marketing application is considered a Breakthrough Therapy marketing application if the sponsor submitting the marketing application is the holder of the Investigational New Drug (IND) for which the Breakthrough Therapy designation was granted, the Breakthrough Therapy designation has not been rescinded, and the marketing application is for the same drug and same indication that was granted Breakthrough Therapy designation. Breakthrough Therapy designation is granted for a specified indication; therefore, the proposed NME indication must be the same indication as, or a subset of, the granted indication. For additional information, see Section 506(a) of the FD&C Act, as added by section 902 of FDASIA.

For drugs initially approved for multiple indications, both breakthrough and non-breakthrough, the subset of indication(s) is for which breakthrough designation was granted is specified.

For drugs approved prior to July 9, 2012 (the date FDASIA was signed into law) breakthrough therapy is listed as “N/A”.

Dosage Form(s)

Definition: Dosage form is the physical manifestation containing the active and inactive ingredients that delivers a dose of the drug product. This includes such factors as [see 21 CFR 314.3(b)]:

(1) The physical appearance of the drug product;
(2) The physical form of the drug product prior to dispensing to the patient;
(3) The way the product is administered; and
(4) The design features that affect frequency of dosing.

Examples of dosage forms include aerosol, capsules, cream, injection, insert, ointment, system, and tablets. For more information on dosage forms see Appendix A in the Draft Guidance for Industry: Product Title and Initial U.S. Approval in the Highlights of Prescribing Information for Human Prescription Drug and Biological Products — Content and Format (January 2018). A product may have several approved dosage forms.

The dosage form listed corresponds to the application number and route of administration displayed in the same order.

The dosage form(s) and route(s) of administration terms in this database are for research purposes only and they may or may not be the same as the approved dosage form(s) or route(s) of administration in the approved labeling. Please see the last approved labeling on Drugs@FDA for the approved dosage form(s) and route(s) of administration.

Fast Track Designation

Definition: A novel drug marketing application is considered a Fast Track marketing application if it is intended, whether alone or in combination with one or more other drugs, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition, or if the drug is designated as a qualified infectious disease product under section 355f(d) of this title.

Fast Track designation is granted for a specified indication; therefore, the proposed NME indication must be the same indication as, or a subset of, the granted indication. Fast Track Designation is regulated under Section 506(b) of the FD&C Act, as added by section 112 of the Food and Drug Administration Modernization Act of 1997 (FDAMA) and amended by section 901 of the Food and Drug Administration Safety and Innovation Act of 2012 (FDASIA).

For drugs initially approved for multiple indications, both fast track and non-fast track, the subset of indication(s) for which fast track designation was granted is specified.

For drugs approved prior to November 21, 1997 (the date FDAMA was signed into law) fast track is listed as “N/A”.

FDA Approval Date

Definition: The FDA Approval Date is the date in which the FDA originally approved the New Drug Application or Biologics License Application that allows commercial marketing of the product in the United States.

FDA Receipt Date

Definition: The FDA receipt date is the date the first and complete marketing application was received by FDA.

Issued a Priority Review Voucher (PRV)

Definition: A Priority Review Voucher is a voucher issued by the Secretary established under either Section 1102 of *FDAAA* titled *Priority Review to Encourage Treatments for Tropical Diseases*, section 908 of the Food and Drug Administration Safety and Innovation Act (FDASIA) titled *Rare Pediatric Disease Priority Review Voucher Incentive Program*, or section 3086 of the 21st Century Cures Act (Cures Act) titled *Encouraging treatments for agents that present a national security threat*.

For NMEs whose applicants received a priority review voucher at the time of approval, this column identifies the program under which the sponsor was eligible to receive a voucher is designated as follows:

- TD – for drugs qualifying to receive a Tropical Disease Priority Review Voucher
- RPD – for drugs qualifying to receive a Rare Pediatric Disease Priority Review Voucher
- MTMC – for drugs qualifying to receive a Material Threat Medical Countermeasure Priority Review Voucher

For drugs approved prior to September 27, 2007 (the date FDAAA was signed into law, establishing the first priority review voucher program), “earned a priority review” is listed as “N/A”.

New Drug Application (NDA) / Biologics License Application (BLA)

Definition: The regulatory pathways for marketing approval of the drug or biological product for human use. A new drug application (NDA) is required for drugs subject to the drug approval provisions of the FDC Act, a biologics license application (BLA) is required for biological products subject to licensure under the PHS Act.

Orphan Drug Designation

Definition: An orphan drug designation identifies whether the sponsor of the drug was granted orphan designation pursuant to section 526 of the Orphan Drug Act for at least one of the initial approved indications. An orphan-designated drug is a drug or biologic intended for the treatment, prevention or diagnosis of a rare disease or condition, which is one that affects less than 200,000 persons in the US or meets cost recovery provisions of the act. The Office of Orphan Products Development reviews requests for orphan-drug designation and will grant the request if all necessary criteria are met.

For drugs initially approved for multiple indications, both orphan and non-orphan, the subset of indication(s) for which orphan designation was granted is specified.

Orphan approvals are based on the Orphan Drug Product designation database. For more information, please see: https://www.accessdata.fda.gov/scripts/opdlisting/oopd/

Proprietary Name

Definition: The proprietary name of a drug product is its brand name (sometimes referred to as the product's "trade name"). Drugs and biological products are not required to have a proprietary name to be marketed. If a drug or biological product does not have a proprietary name, then the field will state "[drug marketed without a proprietary name]."

Qualified Infectious Disease Product (QIDP)

Definition: A qualified infectious disease product (QIDP) identifies whether the approved drug was granted a qualified infectious disease product designation created by Title VIII, section 801, of the Food and Drug Administration Safety and Innovation Act (FDASIA), titled *Generating Antibiotic Incentives Now* (GAIN), under section 505E of the Federal Food, Drug, and Cosmetic Act (FD&C Act). An approved drug product is considered a Qualified Infectious Disease Product if it received QIDP designation and is approved for the use for which the QIDP designation was granted.

For drugs approved prior to July 9, 2012 (the date FDASIA was signed into law) qualified infectious disease product is listed as “N/A”.

Redeemed a Priority Review Voucher (PRV)

Definition: A sponsor of an approved drug redeems a Priority Review Voucher in order to obtain a priority review for the marketing application. A voucher can be exchanged on the open market and transferred to other sponsors.

Drug applications that received a priority review as a result of redeeming a priority review voucher are identified as “Priority (used priority review voucher).”

Review Designation

Definition: Establishes the timeline, milestones, and a goal date by which an application is reviewed under PDUFA performance goals. The review designation can be either standard or priority. The designations Priority and Standard are mutually exclusive.

Priority review reflects the amount of time, or clock, assigned to review an application for a drug that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness of the treatment, diagnosis, or prevention of the serious condition(s) compared to available therapies, **or** an application for a drug that has been designated as a qualified infectious disease product, **or** an application for a drug submitted with a priority review voucher, **or** any supplement that proposes a labeling change pursuant to a report on a pediatric study under 505Ab (only applies to supplements, and therefore had no effect on the designations of applications in the compilation)

Drugs granted priority review based on the redemption of priority review voucher are identified accordingly.

Drugs that do not meet the criteria listed above for Priority review are assigned a Standard review clock.

The review designation recorded in this column is the designation at the time of approval.

Pre-PDUFA Review Designations (1985 – 1991 Approvals) :

The currently in use Priority and Standard review classification system was formalized by Congress in 1992 during the inception of PDUFA. Prior to 1992 a three-tiered classification system grouped drugs into types A (important therapeutic gain), B (modest therapeutic gain), and C (little or no therapeutic gain).Types A and B were the equivalent of todays' priority review, with Type C being the predecessor to the current standard review timeline. The CDER New Molecular Entity and New Biologic Approval Compilation that tracks NME and Original Biologic approvals back to 1985 contains approvals that predate 1992. Review designations that pre-date 1992 were considered priority if they were either a Type A or B class submission. Type C class submissions during this time would be considered as standard.

Route(s) of Administration

Definition: A route of administration is a way of administering a drug to a site in a patient. A product may have several approved route(s) of administration. Examples of common routes of administration include: dental, intra-arterial, intramuscular, intravenous, nasal, ophthalmic, oral, rectal, subcutaneous, topical, transdermal, and vaginal. For more information on commonly used routes of administration in labeling see Appendix B in the Draft Guidance for Industry: Product Title and Initial U.S. Approval in the Highlights of Prescribing Information for Human Prescription Drug and Biological Products — Content and Format (January 2018).

Additional approved supplements/applications for each NME with a new route of administration may have a new application number and may be associated with another dosage form.

The route of administration listed corresponds to the application number and dosage form displayed in the same order.

The dosage form(s) and route(s) of administration terms in this database are for research purposes only and they may or may not be the same as the approved dosage form(s) or route(s) of administration in the approved labeling. Please see the last approved labeling on Drugs@FDA for the approved dosage form(s) and route(s) of administration.