



Bringing New ALS Therapies Forward

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Approval of Radicava

Calaneet Balas, Executive Vice President Strategy

Information on Radicava

- On Friday May 5th, 2017 the FDA approved Radicava for the treatment of ALS.
- This is the first drug approved for ALS in over 22 years.
- Radicava showed a 33% decline in functional disability.
- The drug is expected to be available in the US in August.

Today's Webinar

- Our goal is to provide information on how the FDA approval and insurance coverage processes work for new therapies.
- While The Association does not “endorse” specific products, we do focus on advancing research and development, as well as advocating for access to the best possible care.
- Our co-presenters, Dave Zook and Jason Sapsin, are experienced in these areas and work with The Association.
- Today's webinar is the next in a comprehensive strategy to make sure you have the information needed to understand your options and how to move forward.



FDA Approval

Jason Sapsin, JD

Faegre Baker Daniels

FDA (overview)

- FDA regulates > 20¢ of every \$1 spent by U.S. consumers
- The Agency has enforcement roots
- Has the only federal law enforcement agency exclusively devoted to public health (OCI)
- 16, 738 total employees (FY 2015 Budget Justification)
 - CDRH: 1723 CDER: 4508 ORA: 5250

FDA overview



Structure

Office of the Commissioner
Office of Foods
Office of Global Regulatory Operations and Policy
Office of Medical Products and Tobacco

CFSAN

CVM

CDER

CBER

CDRH

CTP

ORA

OCC



DRUG APPROVALS

FDA Pathway: How Do Drugs get to Market

“Drugs”

Def. Achieve effect through chemical interaction

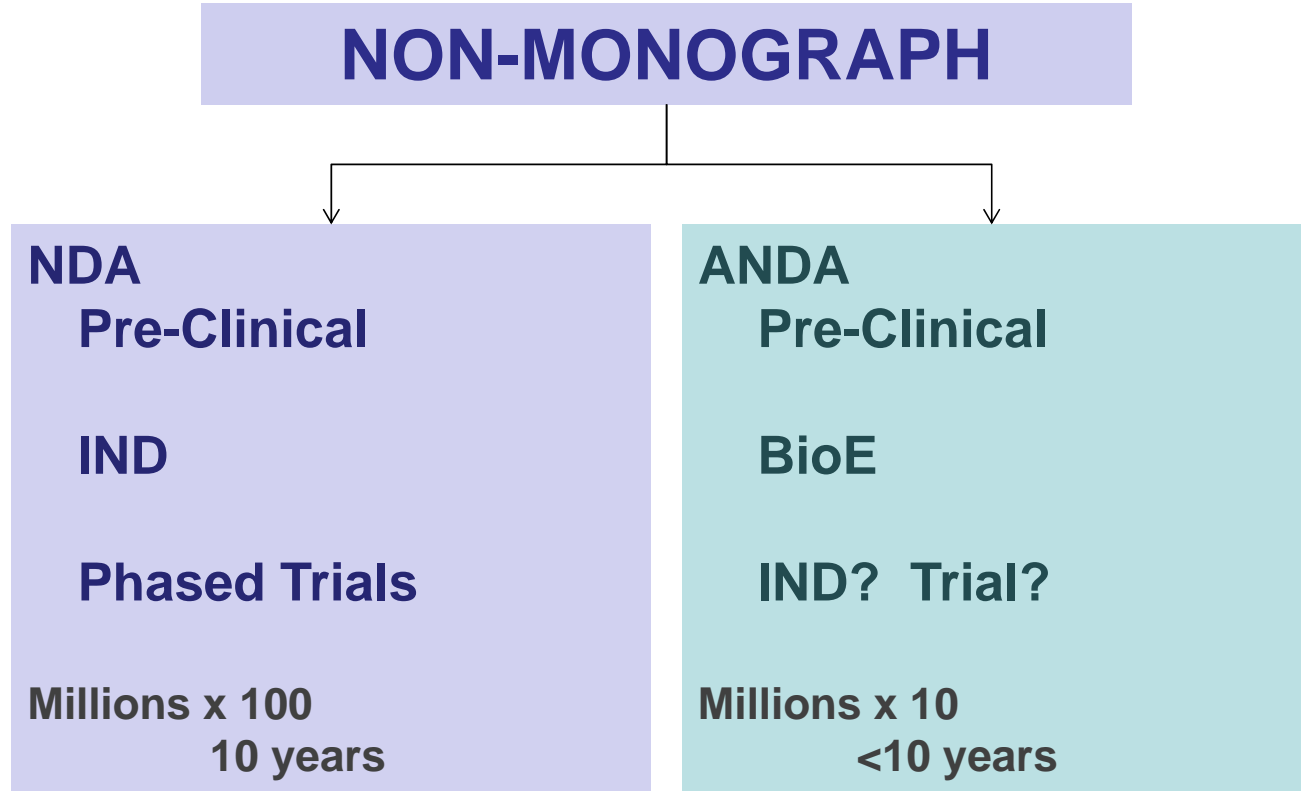
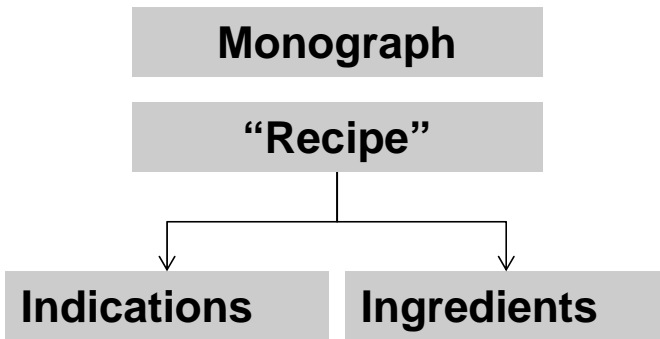
Two ways of thinking about drugs:

- OTC
- Rx

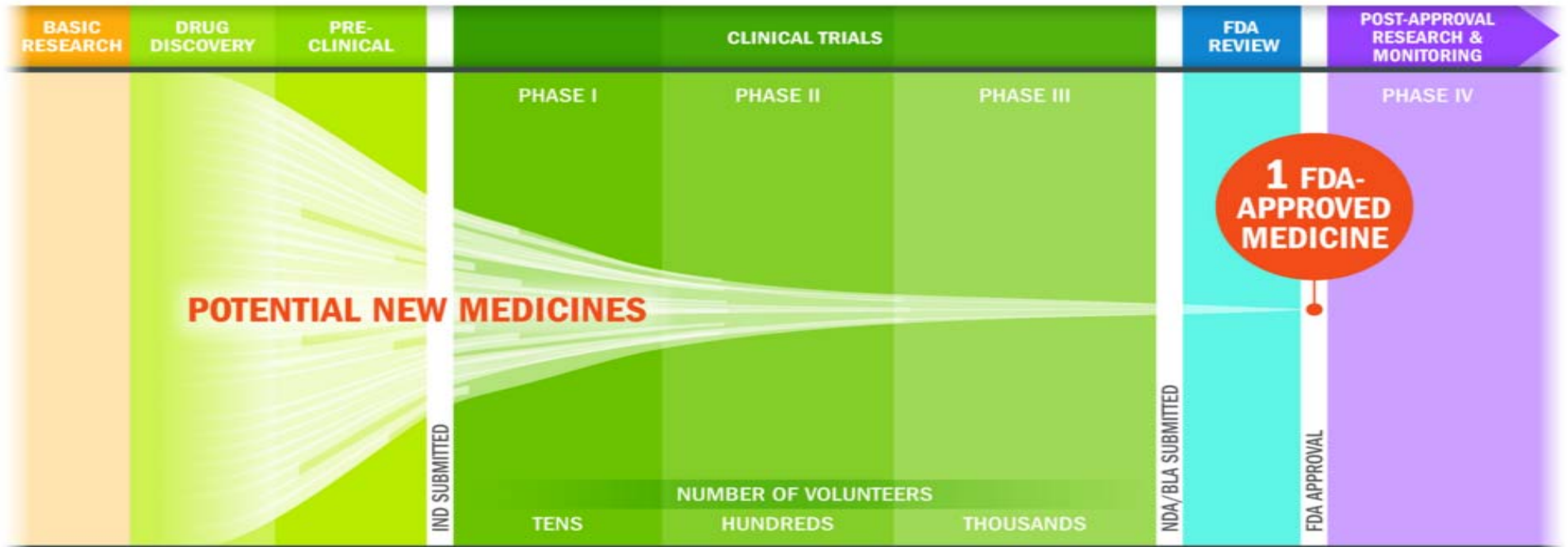
- Monograph
- NDA
- ANDA (generic)

FDA Pathway: How Do Drugs get to Market

Drugs: Pathways



Drug Development Flowchart



SOURCE: <http://www.phrma.org/innovation/clinical-trials>

Regulatory Review Concepts

- Pre-market approval of all drugs (non- Monograph)
 - Aspects of approval
- Indications
- “Off-label”



Production and Distribution Concepts

- ▶ National Drug Code (“NDC”) numbering
 - ▶ Information about NDC numbers is on the FDA’s website at <http://www.fda.gov/cder/ndc/>.
- ▶ Drug manufacturer commences production
- ▶ Reimbursement
 - ▶ CPT codes
 - ▶ Application for new CPT code



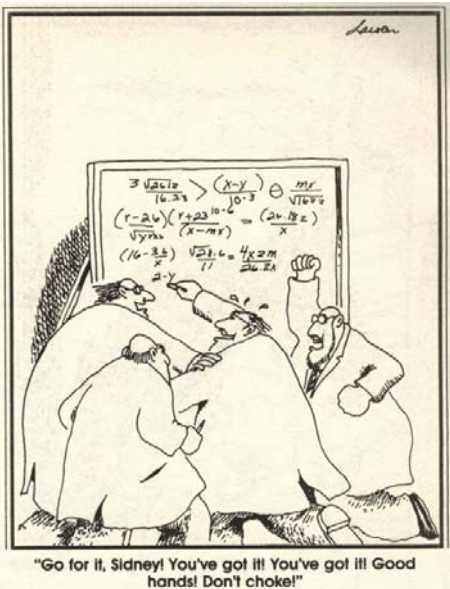
Erdavone

➤ IND
(?)



➤ NDA
➤ Erdavone
submitted /
approved

➤ Approval
➤ Label
➤ Post-market



THE FAR SIDE® By GARY LARSON





Health Insurance Coverage for Newly Approved Drugs

David Zook, JD
Faegre Baker Daniels

Health Insurance Coverage for New Drugs

- Once the drug is approved, gets to physicians and/or pharmacists, and has a code if needed, a health plan will not necessarily automatically pay for it and may need to make a decision about covering the drug or not (and how).

Health Insurance Coverage for New Drugs

Health plans most likely to be involved with covering ALS therapies include:

- private insurance companies (group and individual);
- public insurance programs like Medicaid and Medicare; and
- military health benefits.

Health Insurance Coverage for New Drugs

Health insurance generally has two major components – a medical benefit and a drug benefit – both of which play a role in access to newly approved medications.

- Oral drugs are covered by a drug benefit;
- Injected or **infused drugs** are generally part of the medical benefit; and
- Self-injected drugs fall in both areas depending on the plan.

Health Insurance Coverage for New Drugs

When a new drug is approved, a health plan must decide whether or not to cover it (add it to a health plan's formulary). This takes time.

- Company presents data to Pharmacy Benefit Manager (PBM) and Pharmacy and Therapeutics (P&T) Committee;
- Decision made on whether to include in formulary and what, if any, conditions and exclusions apply.
- Pharmacy & Therapeutics Committees
 - Using medical scientific, economic data, makes recommendations for health plan's list of covered drugs (formulary)
 - Provides input on health plan drug policies, including tiers, cost-sharing, utilization management and coverage determinations

Coverage by Medicare

- Medicare is a federal health insurance program for all US citizens and legal residents who have resided in this country for a continuous period of five years who either:
 - are age 65 and older, or
 - are under age 65 and
 - have received Social Security Disability Insurance (SSDI) (and in some cases, Social Security Insurance) benefits for at least two years, or
 - have been diagnosed with end stage renal disease (ESRD) and who have received kidney dialysis for at least three months or who have received a kidney transplant (Medicare eligibility begins the month of the transplant), or
 - **have amyotrophic lateral sclerosis (ALS, also known as Lou Gehrig's disease) and who receive SSDI benefits (Medicare eligibility begins the month of SSDI eligibility).**

Coverage by Medicare

- Several “Parts” to Medicare for different medical services, products, and programs.
 - Medicare Part B typically pays for FDA-approved injectable and infused drugs given by a licensed medical provider for an FDA-approved indication that is considered reasonable and necessary for treatment and usually isn’t self-administered.
 - In a hospital outpatient department, Part B coverage generally is limited to drugs that are given by infusion or injection.

Coverage by Medicare (cont.)

However, before Medicare pays for some new services or drugs under Part A or B for the first time, Medicare may require a new coverage determination (national or regional) for it.

- Information about National Coverage Determinations (NCDs) and Local Coverage Determinations (LCDs) is on the website of the Center for Medicare and Medicaid Services at <http://www.cms.hhs.gov/DeterminationProcess/>

While Medicare of course covers only Medicare beneficiaries, Medicare coverage policy influences coverage decisions of private insurance as well.

Coverage by Medicare (cont.)

Medicare beneficiaries in Medicare Advantage plans will pay according to the plans for Part B drugs.

- Generally you have to go to a provider in the plan's network to have the plan cover the drug.
- If the drug is a Part B drug, the Medicare Advantage plan must cover it.
- A Medicare Advantage plan that also provides Medicare prescription drug coverage (Part D) can cover B drugs as D if it chooses.

Coverage by Private Insurance

Any policy may have specific exclusions and not cover certain types of care such as all or certain prescription medications.

- Look at your entire policy to see what is covered: do you have drug coverage?
- If so, see what types of drugs are and are not covered.

Coverage by Private Insurance (cont.)

Even if the drug is on the formulary, it may be subject to utilization-management tools such as preferred drug lists with different co-pays or co-insurance requirements, prior authorization, quantity limits, and step therapy.

- “Utilization-management tools” are designed to manage your use of the drug: you go through hoops to get the drug.

Coverage by Medicaid

Medicaid is a cooperative federal/state program that covers health care services for people with limited ability to pay for their health care.

- To qualify for Medicaid, a person must be a resident of the state where applying, a citizen of the U.S. or qualified immigrant, have income below a certain level*, and fit into one of a number of categories of pregnant women and children, caretaker relatives, older individuals, and individuals with disabilities.
- Medicaid typically pays for any FDA-approved drug that the physician prescribes for an FDA-approved indication that you receive as an in-patient or out-patient.
- Some states, however, have recently decided to limit Medicaid's prescription drug coverage and do not cover all drugs for all Medicaid beneficiaries and/or use utilization management tools like private plans.

Appealing Coverage Denials

No matter what kind of insurance you have, if your care (or payment for your care) is denied, you can appeal it.

The rules are different according to the type of insurance you have.

- Medicare
- Medicaid
- Private insurance

Appealing Drug Coverage Denials

- If the drug is not on the plan's formulary, you can ask the plan for an exception.
- If the plan does not give you an exception or it otherwise denies coverage, you can file an appeal.
 - This may be taken care of quickly if the doctor requests an expedited re-determination, but can be a difficult process with several steps.
- If the appeal is denied, you generally can request an external review of some type (including legal reviews for Medicare).



Questions