Compassionate Use, Right to Try, & Access to Unapproved Medicines: Ethical and Practical Issues

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What is Compassionate Use?
Pathway to a New Drug

“compassionate use” – a patient with no other therapeutic options requests to use an investigational product not yet approved for sale and use
FDA Protections
AIDS Activism

IF I DIE OF AIDS - FORGET BURIAL - JUST DROP MY BODY ON THE STEPS OF THE F.D.A.
How to Access Unapproved Drugs
<table>
<thead>
<tr>
<th>Rank</th>
<th>Status</th>
<th>Study</th>
<th>Conditions</th>
<th>Intervention</th>
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<tbody>
<tr>
<td>1</td>
<td>Active, not recruiting</td>
<td>Safety, Toxicity and MTD of One Intravenous IV Injection of Donor CTLs Specific for CMV and Adenovirus</td>
<td>Cytomegalovirus Infection; Adenovirus Infection</td>
<td>Biological: CMV/AdV specific T cells</td>
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<tr>
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<td>Withdrawn</td>
<td>Adenovirus Vaccine Pregnancy Registry</td>
<td>Condition: Adenovirus</td>
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<td>Intervention:</td>
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<td>3</td>
<td>Recruiting</td>
<td>Ad Sensor-based Real-time Diagnosis of Adenovirus</td>
<td>Condition: Adenovirus</td>
<td>Device: Ad sensor</td>
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Seek Compassionate Use/
Expanded Access/Early Access
Understanding Expanded Access/Compassionate Use

Expanded access, also called "compassionate use" is a regulation that makes promising drugs and devices available to patients with serious or immediately life-threatening diseases.

Just as in clinical trials, these investigational drugs/devices have not yet been approved by the FDA and they have not been proven to be safe and effective.

They may be effective in the treatment of a condition, or they may not. It is important to remember that the drug/device may have unexpected serious side effects and that patients need to consider all the possible risks when seeking access to an investigational drug or device.

What You and Your Healthcare Provider Need to Do Before Requesting Expanded Access/Compassionate Use?

Before you or your healthcare provider contact FDA to request Expanded Access to an IND you will need to:

- Search for possible clinical trials you may qualify for by using our [clinical trials search tool](https://clinicaltrials.gov) or visiting
- Search for specific expanded access programs through an online search engine.
- Call drug/device companies directly to ask about their policies.
- Contact patient advocacy organizations to see if they have information on expanded access programs.
- If your healthcare is managed by anyone other than a physician you will need to find a physician who is willing to oversee therapy with a drug/s he is not familiar with, and to work with the company and FDA to obtain the drug, monitor you, and file necessary paperwork. Only a licensed physician is able to apply for expanded access.

Talk with your healthcare provider to see whether use of an investigational drug/device for your treatment is right for you. Be sure to consider how much is known about the investigational drug/device, the severity of your condition, and the likelihood that the therapy will be effective. You and your healthcare provider should consider the kind of illness you have, the stage of disease, other conditions you may be experiencing, and organ function (e.g., liver and kidney function), among other factors.

If you still have questions unanswered or just want to understand the process more contact us, the FDA Office of Health and Constituent Affairs at PatientNetwork@fda.hhs.gov.
no studies found for: DIPG compassionate use

Modify this search | How to Use Search Results

Found no studies with search of: DIPG compassionate use

Recognized Terms and Synonyms:
DIPG compassionate: 0 studies
DIPG: 38 studies
   Diffuse Intrinsic Pontine Glioma
dipinacoline glutamate
Compassionate Use | There Is No Winner
https://maxcurefoundation.org/compassionate-use-no-winner/
Mar 5, 2015 - Alexis lived with DIPG following her diagnosis for a long thirty-three months. ... The problems and perils with the current compassionate use ...

Ayotte Urges FDA to Consider Compassionate Use Request ...
https://www.ayotte.senate.gov/?p=press_release&id...  Kelly Ayotte
Nov 26, 2013 - Ayotte Urges FDA to Consider Compassionate Use Request for ... Lowe was diagnosed with Diffuse Intrinsic Pontine Glioma (DIPG) and was ...

Compassionate Use | Four-Square Gobbies Cancer
4sqgobbiescancer.com/2014/07/30/compassionate-use/  
Jul 30, 2014 - These two incidents occurred and can you believe it, the FDA has a compassionate use program in effect that “should have worked” in both ...

Petition · FDA - Change.org
https://www.change.org/.../fda-save-12-year-old-mckenzie-l...  Change.org
FDA: Save 12-Year-Old McKenzie Lowe; Grant Her Compassionate Use of ... a 12 year old girl with a DIPG brain tumor, a compassionate use exemption to be ...

The Compassionate Use Conundrum | Jonathan Agin
www.huffingtonpost.com/...the-compassionate-use...  The Huffington Post
Apr 23, 2014 - The problem is that the compassionate use program fails to ... known as DIPG in 2008 and outlived the initial prognosis by close to two years.

Family Update: Fall 2014 | Elizabeth's Hope: The Weill ...
elizabethshope.com/updates/family-update-fall-2014/  
He was diagnosed with a diffuse intrinsic pontine glioma (DIPG). ... confirmation from Novartis that the drug would be made available under compassionate use.
The Novartis Declaration for Patients – What Patients Can Expect

Introduction

We are inspired by patients.

This inspiration motivates us to revolutionize the research, development and manufacturing of innovative, high-quality medicines that help people live longer, with a better quality of life, giving more time to do the things that matter to them.

To do our best for patients we do not accept the status quo. We work to enable patient access worldwide so that patients and society can benefit as quickly as possible.

The depth and strength of our pipeline enables us to change the practice of medicine and to bring more breakthroughs with real benefits to patients and society. Our broad portfolio of products helps us to expand overall access to medicines, both through targeted patient access programs and widespread availability of affordable, high-quality generics and biosimilars.

We partner with people and organizations around the world because by working together we can make a greater difference.

We continually challenge ourselves to the highest standards of compliance, integrity and performance in all that we do to ensure a sustainable future of innovation for patients, society and Novartis.

As patients are our focus, it is important that they know what to expect from Novartis and that we are very clear about our commitment to patients and our role and responsibilities in key areas of interest, including:

- Access to our Innovative Medicines
- Patient Safety
- Respecting the Patient Perspective
- Data Transparency and Data Integrity
- Clinical Trial Input
We offer a variety of patient access solutions depending on need and where permitted by law and the country regulatory authorities, including:

- **Early Access Programs** – enables a treatment to be available to patients before it is approved for use on the market and officially launched
- **Expanded Access Programs** – enables a treatment under investigation to be available to patients for treatment outside an ongoing clinical trial
- **Compassionate Use Programs** – enables a treatment to be available to patients who have no treatment options, either because the current available therapies do not work for them or because they have exhausted all of their options, and cannot enter a clinical trial
Bayer: Allow Compassionate Use of Xofigo for Young Mother Fighting Breast Cancer
Margaret Hughes, Brooklyn, NY

My friend Warrior, a mother of two young children, is fighting Metastatic Breast Cancer, and she’s almost run out of options. But a drug Bayer makes called Xofigo (Radium 223) could save her life. Please, ask Bayer to grant Warrior “compassionate use” and allow her to take Xofigo.

Warrior was first diagnosed with breast cancer in 2001. She has tried the chemo drugs that are available to her and undergone extensive radiation, but they have not stopped the cancer from progressing throughout her body. She has even broken her sacrum and been wheelchair bound in an attempt to stop the cancer.

But there is one drug Warrior’s oncologist believes provides significant promise to her – Radium 223. It’s made under the brand name Xofigo by
How to Request Pre-Approval Access to Drugs
no studies found for: expanded access DIPG

Modify this search | How to Use Search Results

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expanded access DIPG: 0 studies
expanded access: 378 studies
DIPG: 38 studies
  Diffuse Intrinsic Pontine Glioma
dipinacoline glutamate
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<tr>
<td>1</td>
<td>Available</td>
<td><strong>Expanded Access /Compassionate Use Protocol For Relapsed Or Refractory CD33 Positive AML Patients In The USA Without Access To Comparable Or Alternative Therapy</strong>&lt;br&gt;<strong>Condition:</strong> CD33 Positive Acute Myelogenous Leukemia&lt;br&gt;<strong>Intervention:</strong> Biological: Antibody Drug Conjugate Chemotherapeutic</td>
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<td><strong>EAP of CPX-351 for Patients 60-75 Years of Age With Secondary AML</strong>&lt;br&gt;<strong>Condition:</strong> Secondary AML&lt;br&gt;<strong>Intervention:</strong> Drug: CPX-351</td>
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<td>3</td>
<td>Completed Has Results</td>
<td><strong>Trial of Decitabine in Patients With Acute Myelogenous Leukemia or Myelodysplastic Syndrome</strong>&lt;br&gt;<strong>Conditions:</strong> Acute Myelogenous Leukemia; Myelodysplastic Syndrome&lt;br&gt;<strong>Intervention:</strong> Drug: Decitabine</td>
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<tr>
<td>4</td>
<td>Available</td>
<td><strong>Expanded Access to T-cell Depleted Haplo-Identical Stem Cells for Patients Receiving Haplo-Identical and Unrelated Cord Blood Transplants</strong>&lt;br&gt;<strong>Conditions:</strong> Hematologic Malignancies; Inborn Errors of Metabolism Disorders; Immune Deficiencies&lt;br&gt;<strong>Intervention:</strong> Biological: ClinIMACS CD34 Reagent System</td>
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Compassionate Use Backgrounder

May 7, 2015

What is “compassionate use”?

Medical professionals use the term “compassionate use” to refer to the treatment of a seriously ill patient with an experimental medicine or product when there are no other treatments available outside of a clinical trial. Other terms used to refer to compassionate use include “expanded access” or “pre-approval access.”

The U.S. Food and Drug Administration (FDA) describes compassionate use as “a pathway for patients to gain access to investigational drugs, biologics and medical devices for serious diseases or conditions. Such investigational drugs/devices have not yet been approved by the FDA and they have not been proven to be safe and effective.”

Most commonly, compassionate use is the term used when a doctor is requesting access for a single patient outside of other company-sponsored programs, like clinical trials or an expanded access program. At Janssen, we use the term “Single Patient Request.”

How does it work?

For an individual patient to receive an investigational medicine or product in the U.S., his or her doctor must request it from both the company developing the therapy and the FDA. FDA regulations impose certain requirements for such requests to be granted. For example, when for investigational medicines Janssen is developing, the best and fastest way to get information about whether or not a patient may be able to gain access is to have the treating physician contact Janssen Medical Information (in the U.S.: 1-800-Janssen or janssenmedinfo@its.jnj.com).

• Providing the investigational drug for the requested use will not interfere with the initiation, conduct, or completion of clinical investigations that could support marketing approval of the expanded access use or otherwise slow the pace of drug development.
• The patient cannot obtain the drug under another clinical trial or expanded access program.

The patient’s physician must also determine that the probable risk to the person from the investigational medicine is not greater than the probable risk from the patient’s disease or condition. Beyond these requirements of FDA regulation, companies developing therapies may consider other factors in considering whether to grant requests for individual access, such as the availability of supplies of the investigational medicine.

How can a patient request access?

In addition to the patient, there are three key people or groups involved in the compassionate use process: the patient’s doctor, the FDA, and the company investigating the medicine.

• The doctor must review all of the FDA requirements for expanded access to the investigational treatment with the patient and obtain informed consent.
• The doctor may need to get Letter of Authorization (LOA) from a representative of the company investigating the treatment.
• The doctor will need to complete an application with the FDA requesting access to the investigational medicine, including an LOA as part of this application allows the FDA to refer to the official investigational new drug (IND) application for that medicine when reviewing the doctor’s request.

For investigational medicines Janssen is developing, the best and fastest way to get information about whether or not a patient may be able to gain access is to have the treating physician contact Janssen Medical Information (in the U.S.: 1-800-Janssen or janssenmedinfo@its.jnj.com).
Release Brincidofovir for compassionate use to treat the adenovirus plaguing 7-year old Josh Hardy right now.

Regina Breedlove
Mechanicsville, VA

When Josh, my cousin's son, was 9 months old, he was diagnosed with a malignant rhabdoid tumor of the kidneys. It's a highly aggressive, rare form of cancer that only 15 children are diagnosed with each year. In November 2013, he had a bone marrow biopsy and
How to Access Unapproved Drugs

• Join a clinical trial

• Have knowledgeable doctors, resources, and contacts; ask a pharmaceutical company; have the time and know-how to apply pressure if company says no

• Legislative means?
“Right to Try” Laws

An Act

HOUSE BILL 14-1281


CONCERNING THE ALLOWANCE FOR TERMINALLY ILL PATIENTS TO HAVE ACCESS TO INVESTIGATIONAL PRODUCTS THAT HAVE NOT BEEN APPROVED BY THE FEDERAL FOOD AND DRUG ADMINISTRATION THAT OTHER PATIENTS HAVE ACCESS TO WHEN THEY PARTICIPATE IN CLINICAL TRIALS.
Frequently Asked Questions (FAQ)
About Compassionate Use and Pre-Approval Access

What is “compassionate use”?
Compassionate use grants individuals access to a drug or device that hasn’t been approved for sale or use in the United States by the Food and Drug Administration (FDA). Normally patients in the U.S. have access to such unapproved medical products only by enrolling in a clinical trial. Compassionate use does not involve enrolling in a clinical trial. (This is a little complicated, and we discuss it more thoroughly below.) Terminally ill patients who have no other treatment options may seek this type of access—which is also known as compassionate access, expanded access, single- or individual-patient access, and, more recently, pre-approval access. So might those with debilitating...

Outside the U.S., policies for pre-approval use (also known as “named patient programs,” “named patient supply,” or “expanded access programs”) vary by country. This FAQ pertains only to the United States.

What is an unapproved medical product?
In the U.S., medicines and medical devices are available for sale or use only after they are approved by the FDA, the federal agency charged with making sure medical products have been shown to be relatively safe and effective before they can be placed on the market. (If evidence arises later that a medical product is not as safe or effective as thought, the FDA can withdraw it from the market.)