The BROAD BENEFITS *of* AIDS RESEARCH

Many new treatments for diseases such as cancer, kidney and heart disease, COVID-19, and hepatitis have arisen from research on HIV/AIDS.

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MAKING AIDS HISTORY


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Since the beginning of the epidemic, AIDS research has been a testing ground for new concepts and technologies in drug development, diagnostics, and disease prevention. Drugs developed to combat HIV and to treat AIDS have helped improve and prolong the lives of countless people worldwide.

But discoveries made in one area of research often benefit the study and treatment of a wide variety of human diseases.

The fact is that many new treatments for diseases such as cancer, hepatitis, heart and kidney disease, and COVID-19 have arisen from research aimed at preventing, diagnosing, and treating HIV/AIDS.

"The HIV/AIDS research model has proven that cross-fertilization of ideas, innovation, and research progress can lead to unforeseen and substantial advantages for a variety of other diseases."

–Anthony Fauci, MD, Former Director, National Institute of Allergy and Infectious Diseases

Are there serious diseases unrelated to HIV that have been put into REMISSION or even CURED as a direct result of AIDS research?

Absolutely, and some recent examples have been very dramatic. In 2012, a young girl with **acute leukemia** wasn't responding to conventional treatments and was on the brink of death. But then she was given an experimental treatment using an anti-cancer gene that could only be delivered to her cells using disabled HIV as a carrier. Within weeks she was in remission and she remains cancer free.

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In 2014, a Utah man with the same type of aggressive leukemia received a similar treatment using disabled HIV (known as the SIN vector) and he, too, is now cancer free. Most recently, the SIN vector has been used in the cure of sickle cell anemia in young people with severe disease.

Additionally, six HIV-negative children with usually fatal **genetic immune disorders** (Wiskott Aldrich syndrome, severe combined immunodeficiency, and chronic granulomatous disease) were cured using similar targeted gene therapies, all of which employed inactivated HIV.


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For their groundbreaking work on mRNA that led to the rapid development of effective vaccines for COVID-19, amfAR grantee Dr. Drew Weissman and collaborator Dr. Katalin Karikó were awarded the 2023 Nobel Prize in Physiology or Medicine.

How did HIV research help scientists develop COVID-19 VACCINES in record time?

The rapid development of effective COVID-19 vaccines would not have been possible without HIV vaccine research.

All leading COVID-19 vaccines and vaccine candidates to date, including Pfizer and Moderna, rely on their ability to stimulate production of antibodies and T cells targeted to a COVID-19 virus coat protein known as S, or "spike." S is required for the virus to infect a cell. But to ensure that a vaccine elicits an optimal response, its S protein component must be engineered to ensure that it is stabilized in the correct shape. This relies on insertion of an amino acid called proline into specific parts of S—a technique developed many years ago in the course of HIV vaccine research.

For the Pfizer and Moderna vaccines, delivery of the COVID-19 vaccine products starts with a genetic material, mRNA, encoding the optimized S protein. For over three decades, mRNAs have been tested as vaccine and therapeutic candidates, but with little success for two important reasons. First, when mRNA is delivered to cells, it induces a dangerous and potentially lethal immune response. To circumvent this problem, a technique for its modification was developed by Dr. Drew Weissman, a long-time amfAR grantee.

Once the mRNA is modified for safety, it must still be delivered to cells, and the second challenge is that mRNA is very fragile. Without specialized lipid shells, known as nanoparticles or "fat bubbles," to protect it from being rapidly dissolved in tissue, and to facilitate its entry into immune cells, there would be no mRNA COVID-19 vaccine. Among their earliest therapeutic applications, these lipid nanoparticles were used for the evaluation of this mRNA/lipid strategy to produce anti-HIV antibodies in mice.

How is AIDS research helping people with CANCER?

Experimental treatments for several types of cancer have grown directly out of AIDS research. Treating HIV involves blocking key receptors—proteins used by the virus to enter immune cells. One of these receptors, CXCR4, appears to be an important target in treating **lung cancer.** Researchers are now studying drugs originally designed to block CXCR4 in HIV patients to determine whether they might be used to fight this common form of cancer.

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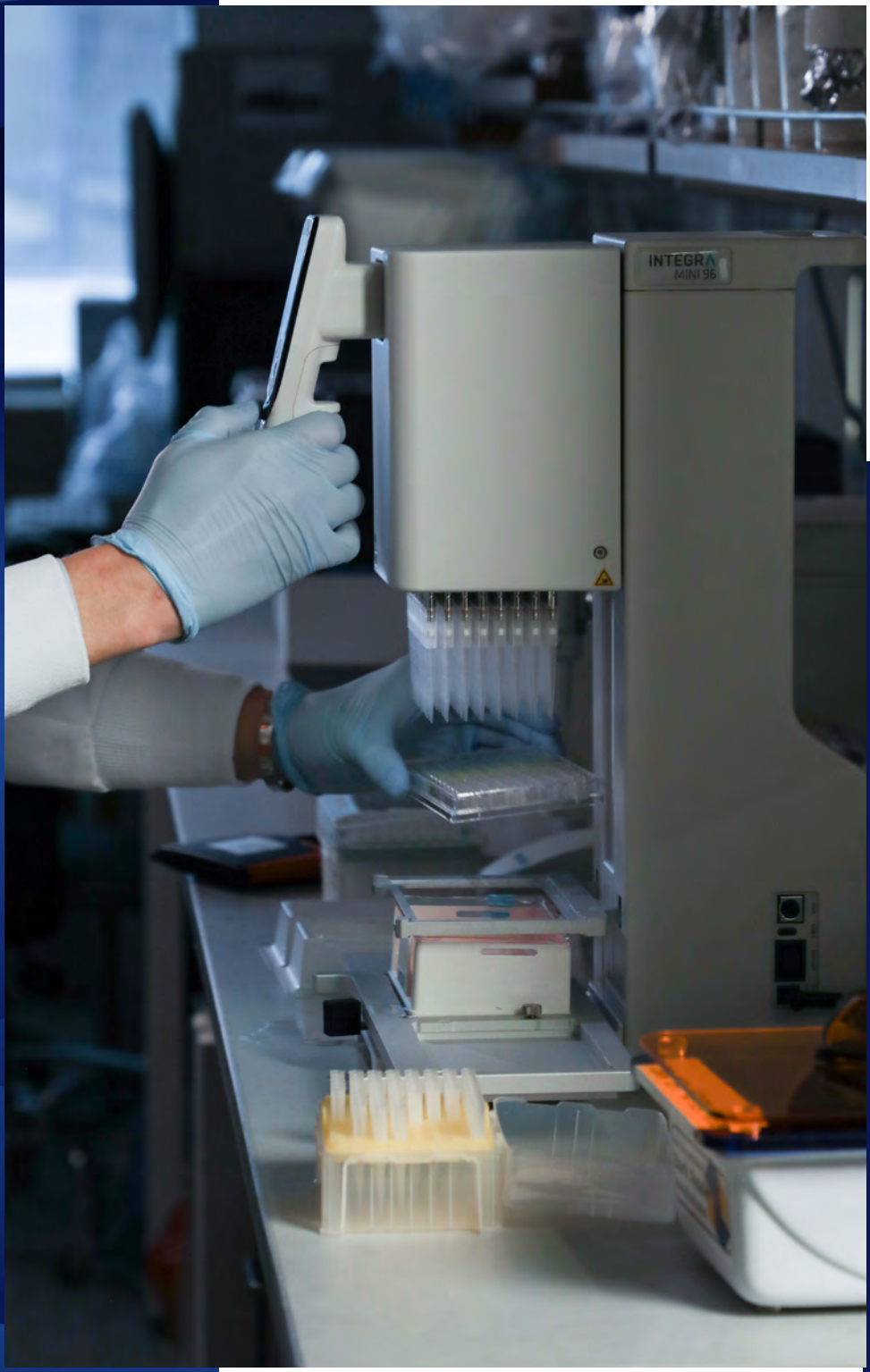
Several natural body hormones called growth factors promote the activity of HIV. Many of these hormones also accelerate the growth and spread of cancer cells. Blocking the activity of these hormones is a strategy first used experimentally to treat Kaposi's sarcoma, a cancer found in patients with HIV/AIDS. Now it is also being tested in **bladder, vulvar,** and **breast cancers,** and has shown some exciting recent success in treating **colon cancer.**

In addition, small proteins and drugs that can block the growth of new blood vessels (which is critical to the survival of tumor cells) were originally developed to treat Kaposi's sarcoma, but are now being tested in many other cancers as well. Finally, the protease inhibitor lopinavir, first developed to treat HIV, has been shown to be effective in attacking human papillomavirus, which can cause **cervical cancer.**

The profound immune suppression necessary for a bone marrow transplant to treat **leukemia** and other cancers often leads to devastating, even fatal, infections such as cytomegalovirus and *Pneumocystis* pneumonia, which also affect people with AIDS. New drugs to treat and prevent these infections in cancer patients have come directly from AIDS-targeted research.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Have HIV DRUGS been used in the treatment of other infectious diseases?

Yes. HIV/AIDS therapies are critical in the treatment of other diseases. The HIV protease inhibitor, ritonavir, is one of the two main ingredients in Paxlovid, the primary antiviral drug used to treat COVID-19. Protease inhibitors, one of the first classes of anti-HIV drugs, revolutionized treatment for people living with HIV/AIDS in the mid-1990s and contributed to a rapid decline in AIDS-related deaths.

Three drugs developed to treat HIV—lamivudine, tenofovir, and entecavir—are now the mainstays of therapy for **hepatitis B virus** (HBV) infections. Another antiviral drug called adefovir, which failed as an HIV treatment, was found to suppress HBV at much lower dosages and has been approved for the treatment of chronic HBV disease. More recently, a drug called sofosbuvir that is modeled on HIV reverse transcriptase inhibitors, one of the main classes of anti-HIV drugs, is being used to treat and cure **hepatitis C.**

Since HIV is a virus that attacks the immune system, what does AIDS research teach us about AUTOIMMUNE DISORDERS or immune-based therapies for other diseases?

HIV-positive people may develop autoimmune problems, such as **psoriasis** or blood abnormalities associated with **lupus**. For these autoimmune diseases, treatments developed for HIV/AIDS should also apply when the same conditions occur spontaneously. A class of anti-HIV drug that blocks a protein known as CCR5, the key co-receptor for HIV's entry into cells, is also being evaluated in **inflammatory bowel disease** and other autoimmune disorders.

Certain hormones that modify the function of immune cells are now being tested as treatments for HIV. Some of the most recent include IL-12 and TNF (tumor necrosis factor)-alpha inhibitors, which may also boost the immune systems of **cancer** patients. In those patients, the hormones help destroy the residue of cancer after surgery, radiation, or chemotherapy. The TNF-alpha inhibitors may also be useful in combating the **body wasting** that accompanies AIDS and some forms of cancer.

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How are new technologies developed for AIDS research advancing the DIAGNOSIS landscape?

One particle of HIV genetic material can be located among millions of other particles by using extraordinarily sensitive techniques known as PCR (polymerase chain reaction) and RT-PCR (reverse transcription PCR).

New PCR tests, developed for diagnosing HIV, are now routinely used to rapidly detect a number of other infectious diseases, including **COVID-19, hepatitis C, tuberculosis, chlamydia, influenza, Lyme disease**, and many **fungal infections.**

These techniques have also made it possible to measure otherwise undetectable levels of **cancer** cells in people who appear to have been cured. This detection allows doctors to initiate new therapy or to continue ongoing treatments that might otherwise have been discontinued.

Further, the discovery of HHV-8, a **herpes virus** linked to Kaposi's sarcoma, was made possible by a new application of PCR. This technique is now being used worldwide to seek possible infectious causes for diseases of unknown origins.

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Also, studying immune dysfunction associated with HIV has driven advances in affordable, automated, and portable point-of-care devices that could have a range of clinical applications in resource-limited settings.

Apart from the science, how else is amfAR's work HELPING PEOPLE with illnesses other than AIDS?

Advocacy efforts by amfAR and other organizations were instrumental in getting the Food and Drug Administration to institute fast-track procedures to speed the review of new treatments for all life-threatening diseases. Fast-tracking has already been applied in the approval of treatments for conditions such as **Alzheimer's, AIDS,** and **cancer.**

In summary, AIDS research is providing insights into a range of diseases, their causes, and their treatment. Better diagnostic methods, therapies to restore the immune system, newer preventive antibiotics and drugs, and new treatments for infectious diseases and cancer–all developed in the course of AIDS research–are improving and prolonging countless lives every day.

Learn more about the latest in amfAR-funded HIV/AIDS research programs by visiting us at www.amfar.org. For more information please contact us at (800) 39-amfAR or at donors@amfar.org.

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