

Issue of Serious Concern: America's Next Generation of Transplant Drugs

Agency: U.S. Food and Drug Administration (FDA)

- Bipartisan American kidney policy (under Presidents Obama, Trump, and Biden) has prioritized kidney transplantation as the preferred therapy, versus dialysis, for Americans suffering from kidney failure. The 2016 Obama White House Organ Summit, the 2019 Trump Executive Order on Advancing American Kidney Health, and President Biden's recent signing of a bipartisan bill to drive competition in the American organ allocation system all support increased kidney transplantation.
- However – the development of new, immunosuppressive drugs to maintain kidney transplants, increase long-term survival, and reduce the need for a second transplant has not kept pace with national transplant policy priorities. AAKP conducted a national survey of over 1,200 kidney transplant patients, organ donors, and transplant professionals in 2023 that highlighted the concerns of kidney transplant patients and organ donors about this unmet need.
- **Inaction by the U.S. FDA is a major roadblock to the next generation of transplant drugs. Congress must closely monitor the lack of alignment between FDA transplant drug development initiatives and bipartisan, national transplant policy. For over 15 years, transplant surgeons, patients, and industry have met with the FDA to discuss the need for new drugs, with less side effects, that help transplanted kidneys last longer.**
- Current drugs have serious side effects including hypertension, cardiovascular disease, gastrointestinal issues, skin cancer, and other serious cancers. **Kidney transplant patients require constant monitoring because status quo transplant drugs can be toxic to the donated kidney (nephrotoxicity). This causes organ failure, a return to dialysis, and need for a second transplant – or premature death.**

America's Next Generation of Transplant Drugs continued...

- Over **20%** of the kidney patients on the organ waiting list are waiting for **a second kidney/re-transplant**. Many patients die waiting for a second transplant.
- Status quo immunosuppressive transplant drugs were developed based on clinical endpoints that date back 20 and 40 years. These drugs were approved by the FDA on a clinical endpoint of 1-year graft survival – meaning the kidney transplant lasted 1 year. Today, 1-year graft survival exceeds 95% for most transplanted kidneys due to evolutions in transplant science and technique.
- **FDA has held 4 major public meetings in the past 8 years on this issue – with no action. FDA is holding its 5th meeting on the issue of transplant drug development on November 9th, 2023 – we encourage Congressional staff to listen to this meeting.** Patients and transplant surgeons will report back to Congress on what they hear at this meeting.
- In 2018, FDA asked transplant experts and industry to create new transplant clinical endpoints through the FDA-backed Critical Path Institute and the Transplant Therapeutics Consortium (TTC). **After 5 years of great effort – involving FDA technical advisors, patients, top researchers, industry, and transplant surgeons - TTC submitted a new, proposed co-primary clinical endpoint to the FDA on July 31, 2023. The FDA may make a final decision on the TTC application for a new co-primary clinical endpoint for transplant drug development as early as the first quarter of 2024.**

America's Next Generation of Transplant Drugs continued...

- **If the FDA fails to approve the new TTC co-primary clinical endpoint for transplant drugs, AAKP believes Congress should ask the Government Accountability Office (GAO) to review the FDA decision, hold a public hearing, and review holdups within the FDA regarding new transplant drug development and its potential impact on national kidney transplant policy and long-term patient outcomes.**

Supplemental Material:

- [National Survey Results](#) on the “Future of Transplant Drug Innovation,” an independent survey conducted under the AAKP Center for Research and Education.
- AAKP Action Center Petition “Transplant Therapeutics Must Evolve!” <https://bit.ly/TransplantTherapeutics>.
- FDA Public Workshop, “Endpoints and Trial Designs to Advance Development in Kidney Transplantation.” [Register by clicking here](#).

