

April 5, 2021

***VIA ELECTRONIC DELIVERY***

Commissioners & Staff  
Medicaid and CHIP Payment and Access Commission (MACPAC)  
1800 M St NW, Suite 650 South  
Washington, DC 20036

**RE: Comments on MACPAC's Draft Recommendations on Accelerated Approval Drugs**

Dear MACPAC Commissioners & Staff,

The Institute for Gene Therapies (IGT or “the Institute”) submits these comments to the Medicaid and Children’s Health Insurance Program (CHIP) Payment and Access Commission (MACPAC or “the Commission”) regarding two draft recommendations put forth to the Commission regarding differential rebates for accelerated approval drugs. IGT is concerned with and opposes the draft recommendations put forth by the Commission. The Institute believes that if the draft recommendations are adopted, patient access to new therapies, including gene therapies, could be significantly delayed and cause a detrimental impact on patient quality of life.

IGT was launched in February of 2020 to advocate for a modernized regulatory and reimbursement framework that encourages the development of transformative gene therapies and promotes patient access. Through a Corporate Advisory Council, Patient Advocacy Advisory Council, and Scientific, Academic & Medical Council, the Institute represents a wide array of patient advocacy groups, gene therapy manufacturers, and scientific, medical, and academic stakeholders seeking to advance the promise of gene therapies. IGT aims to inform the conversation regarding the value of transformative therapies and advocate for policies and practices to ensure patient access to these treatments. A full list of our members is available at <https://www.gene-therapies.org/advisory-councils>.

**IGT opposes the draft recommendations for four primary reasons outlined below:**

1. The draft recommendations don’t accurately consider the purpose and standards of the Food and Drug Administration’s (FDA’s) accelerated approval pathway.
2. MACPAC is exceeding its statutory authority and attempting to solve a misunderstanding of the FDA’s accelerated approval pathway by increasing Medicaid drug rebates, which could lead to unintended consequences for Medicaid beneficiaries.
3. The draft recommendations, if adopted by Congress, would stifle innovation and delay new therapies while providing inconsequential savings to the Medicaid program.
4. MACPAC has not conducted an analysis or projection of the impact of the draft recommendations, which could be adopted by Congress to help pay for other, unrelated legislation to the detriment of Medicaid beneficiary access to care.

**1. The draft recommendations don't accurately consider the purpose and standards of the FDA's accelerated approval pathway.**

In 1992, the FDA established the accelerated approval pathway to enable the approval of drugs for serious or life-threatening conditions that filled an unmet medical need.<sup>1</sup> The pathway was established in response to the growing HIV/AIDS epidemic and to provide access to cancer treatments. Approval is based on a surrogate endpoint, which is a marker (e.g., laboratory measurement, radiographic image, physical sign, or other measure) that is reasonably likely to predict clinical benefit.<sup>2</sup> Using a surrogate endpoint can save valuable years in the drug approval process for patients facing serious or life-threatening illnesses and there is often extensive dialogue between sponsors and FDA prior to an accelerated approval decision. Recognizing the value of accelerated approval, in 2012, Congress authorized the FDA to use the pathway to approve drugs treating rare diseases.<sup>3</sup> All drugs approved via the accelerated approval pathway meet the FDA's gold standard for safety and efficacy and not considered experimental, investigational, or low evidence – that is, they must meet the same statutory standards for safety and effectiveness as drugs granted traditional approval.<sup>4</sup>

Drug companies must still confirm the anticipated clinical benefit of a drug approved under accelerated approval through phase 4 confirmatory trials. If the confirmatory trial demonstrates clinical benefit, the drug is granted traditional approval by the FDA. If the confirmatory trial fails to verify clinical benefit, the FDA may withdraw approval.<sup>5</sup>

As of December 31, 2020, drugs and biologics impacting 253 indications had received approval under the FDA's accelerated approval pathway.<sup>6</sup> These drugs treat serious and life-threatening conditions such as AIDS, cancer, and rare diseases impacting children and adults. Because of the FDA's accelerated approval pathway, each of these treatments has been made available to patients years earlier than otherwise would have been permitted. FDA works with sponsors to determine whether or if an accelerated approval drug should be withdrawn and recent activity by FDA and sponsors has led to several withdrawals.<sup>7</sup>

**2. MACPAC is exceeding its statutory authority and attempting to solve a misunderstanding of the FDA's accelerated approval pathway by increasing Medicaid drug rebates, which could lead to unintended consequences for Medicaid beneficiaries.**

MACPAC is charged with reviewing the policies of the Medicaid program that affect access to covered items and services under Medicaid (e.g., payment, eligibility, enrollment and retention, benefit and coverage, and quality).<sup>8</sup> MACPAC is not charged with recommending policies to affect the impact of FDA programs. Unfortunately, MACPAC appears to be addressing a perceived lack of incentive for drug manufacturers to complete post-accelerated approval confirmatory trials, while presenting no evidence to support this assertion. Staff presentations presume that increasing the Medicaid drug rebate will serve as an incentive for manufacturers to complete these trials.

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<sup>1</sup> 57 Fed. Reg. 58959 (Dec. 11, 1992) (codified at 21 CFR pt. 601 subpt. E).

<sup>2</sup> 21 C.F.R. § 601.41.

<sup>3</sup> Food and Drug Administration Safety and Innovation Act of 2012, Pub. L. No. 112-144, 126 Stat. 993-1132 (2012).

<sup>4</sup> Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologicals (May 2014), available at: <https://www.fda.gov/media/86377/download> (last accessed April 1, 2021).

<sup>5</sup> 21 C.F.R. § 601.43.

<sup>6</sup> “CDER Drug and Biologic Accelerated Approvals Based on a Surrogate Endpoint,” available at: <https://www.fda.gov/media/88907/download> (last accessed March 26, 2021).

<sup>7</sup> See <https://www.raps.org/news-and-articles/news-articles/2021/3/industry-wide-accelerated-approval-review-yields-f>.

<sup>8</sup> Social Security Act § 1900(b).

IGT recognizes the challenges when companies do not complete confirmatory trials. However, there are often valid clinical reasons why confirmatory trials may be delayed (e.g., identification of eligible patients, manufacturing/supply chain constraints, competing for patients with other trials) and there is a different pathway to address these concerns. Moreover, MACPAC's proposed recommendations do nothing to distinguish those instances in which post-accelerated approval confirmatory trials are delayed for scientifically valid reasons. In fact, we note that there is no such way to take into account this reality because MACPAC was not established to have the expertise to do so nor is the rebate mechanism in the Medicaid Drug Rebate Program sufficiently nuanced or appropriate to pull through such a distinction. The authority over the FDA accelerated approval process, including the completion of confirmatory phase 4 trials, lies solely with the FDA and not MACPAC.<sup>9</sup>

This discussion, and the related recommendations, were borne from MACPAC's analysis of high-cost specialty drugs, the pipeline for such drugs over the next 3-5 years, and the challenges for Medicaid to manage these drugs. In fact, the only "challenge" listed by MACPAC staff as it relates to accelerated approval is "limited evidence" and not completion of confirmatory clinical trials.<sup>10</sup> Again, as described above, the FDA created the accelerated approval pathway to enable the approval of drugs for serious or life-threatening conditions that fill an unmet medical need using a surrogate endpoint. The FDA can only grant approval for a drug under this pathway based on adequate and well-controlled clinical trials establishing that the drug has a surrogate endpoint that is reasonably likely to predict clinical benefit.<sup>11</sup> These regulations, and the accelerated approval pathway, have been so effective at bringing needed therapies to market, that they have not been modified since their adoption in 1992.<sup>12</sup> Rather, as noted above, Congress expanded the pathway to rare diseases in 2012 through the Food and Drug Administration Safety and Innovation Act of 2012.

**3. The draft recommendations, if adopted by Congress, would stifle innovation and delay new therapies, while providing inconsequential savings to the Medicaid program.**

Accelerated approval is used to authorize and provide life-saving treatments to patients who have limited or no other treatment options and suffer from serious and/or life-threatening diseases years in advance of the timeframe required under the traditional FDA approval process. A full trial – which could last the better part of a decade – will unnecessarily rob patients of access to medical advances that could otherwise have provided the chance to slow the pace of their disease or halt progression altogether; this reality could differentially and negatively impact the pediatric population given the diseases for which therapies are being currently developed through the accelerated approval pathway. As MACPAC staff noted in presentation materials from the March 2021 public meeting, these draft recommendations may disincentivize manufacturers from seeking accelerated approval for products due to an increased rebate while confirmatory trials are being completed.<sup>13</sup>

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<sup>9</sup> Note: FDA has the authority to remove a drug if it does not confirm benefit in the confirmatory trial. See 21 C.F.R. § 601.43(a)(1).

<sup>10</sup> MACPAC January 2021 meeting presentation, "Payment and Coverage of High-Cost Specialty Drugs Report from Technical Advisory Panel," available at: <https://www.macpac.gov/wp-content/uploads/2021/01/Payment-and-Coverage-of-High-Cost-Specialty-Drugs-Report-from-Technical-Advisory-Panel.pdf> (last accessed March 26, 2021).

<sup>11</sup> 21 C.F.R. § 601.41.

<sup>12</sup> Two technical corrections have been made, see 68 Fed. Reg. 34797 (June 11, 2003) and 70 Fed. Reg. 14984 (Mar. 24, 2005).

<sup>13</sup> MACPAC March 2021 meeting presentation, "High-Cost Specialty Drugs: Moving Towards Recommendations," available at: <https://www.macpac.gov/wp-content/uploads/2021/03/High-Cost-Specialty-Drugs-Moving-Towards-Recommendations.pdf> (last accessed March 26, 2021).

MACPAC bases part of its rationale for the draft recommendations as a solution to the problem that high-cost specialty drugs present to the Medicaid budget. This is a solution in search of a problem, however. A recent analysis of Medicaid spending from 2007-2018 shows that accelerated approval drugs accounted for less than 1% of annual Medicaid spending.<sup>14</sup> Between 2007 and 2018, total Medicaid spending increased an average of 5.7% per year; accelerated approval drugs accounted for only 1.3% of this growth (compared to 29% for hospital care and 16.2% for physician services).<sup>15</sup> Drugs approved under the accelerated approval pathway meet an urgent and unmet medical need, often addressing conditions that disproportionately impact the pediatric and low-income populations Medicaid serves. As Thorpe and Holtz-Eakin conclude, “Despite cost concerns, the analysis on drivers of Medicaid spending shows that accelerated approval drugs have a de minimis impact on spending while addressing significant unmet medical needs.”<sup>16</sup>

**4. MACPAC has not conducted a thorough analysis or projection of the impact of the draft recommendations, which could be adopted by Congress to help pay for other, unrelated legislation to the detriment of Medicaid beneficiary access to care.**

The draft recommendations do not specify the actual increased rebate or inflationary rebate that MACPAC would be recommending. As such, commissioners have not been presented with sufficient analysis regarding the potential impact of these recommendations on Medicaid enrollee access to life-saving therapies. Commission staff even notes that beneficiaries may lose access to some products, a detrimental effect that must be analyzed fully before MACPAC makes such recommendations. As cited earlier, Medicaid spending on accelerated approval drugs represents such a small share of overall Medicaid spending that the detrimental impact on manufacturers’ pursuit of these life-savings drugs does not justify the insignificant impact such cuts would have on Medicaid spending. MACPAC is charged with reviewing Medicaid policies that affect access to covered items and services. These recommendations would only serve to restrict access.

**Conclusion**

The Institute appreciates the opportunity to submit comments to MACPAC on the draft recommendations regarding a differential rebate model for accelerated approval drugs. We affirm our opposition to these recommendations, urge MACPAC to withdraw the draft recommendations for further analysis, and hope that MACPAC will continue to examine policies to increase access to life-saving therapies rather than addressing FDA’s accelerated approval process. IGT would be pleased to answer any questions regarding the issues raised in these comments and we are happy to serve as a resource on gene therapy issues in 2021 and beyond.

Sincerely,



John R. Feore, III  
Director, Health Policy

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<sup>14</sup> Thorpe, PhD, Kenneth E. and Holtz-Eakin, PhD, Douglas, “Limiting Medicaid Access to Accelerated Approval Drugs: Costs and Consequences,” AJMC Article (Mar. 30, 2021), available at: <https://www.ajmc.com/view/limiting-medicaid-access-to-accelerated-approval-drugs-costs-and-consequences> (last accessed April 1, 2021).

<sup>15</sup> Id.

<sup>16</sup> Id.