

**Don't put the CAR-T before the Horse:  
Proper Planning for Novel Gene Therapies in an Uncertain Regulatory Environment**

by

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I. History of Cell and Gene Therapy

- A. **1970s.** In 1971, DNA inserted into a gene in vitro and restored defective enzyme activity. In 1972, a formal proposal for gene therapy is published in the journal *Science*.
- B. **1980s.** Clinical trials commence on humans involving use of viruses to alter cells genetically.
- C. **1990s.** Scientists use gene therapy to cure ADA-SCID disease, a genetic disorder creating immuno-deficiency, aka “bubble boy” syndrome. Separately, in 1999, University of Pennsylvania’s experimental gene therapy for Jesse Gelsinger causes his death.
- D. **2000s.**
  - 1. In 2003, China approved the first gene therapy for head and neck squamous cell carcinoma, called Gendicine.
  - 2. In 2010, Provenge is approved for prostate cancer. Provenge extracts and multiplies a patient’s own white blood cells. It does not involve genetic modification.
  - 3. In 2012, Glybera was approved by the EU to cure a protein deficiency leading to pancreatitis. At a cost of \$1.6 million, only one patient was treated with the drug after the close of the clinical trial.
- E. **2017.** In August, Novartis’ Kymriah is approved for pediatric population. In October, Kite’s Yescarta is approved.

- F. **2018.** Kymriah is approved for adults.
  - G. Tests remain ongoing for genetic deficiencies, cancers, and some viruses.
- II. Chimeric Antigen Receptor T-Cell Therapy – A form of Cell and Gene Therapy
- A. What is CAR-T?
    1. The patient’s own blood-based T-cells, a form of cells that are part of the human immune system, are collected via apheresis.
    2. The cells are genetically engineered by using viral vectors to introduce receptors called Chimeric Antigen Receptors (or CARs) on the cell surface.
    3. These modified cells are expanded or grown in vitro to a volume appropriate to reinfuse back into the patient.
    4. The CARs on the cells are designed such that they seek out a certain protein (or antigen) on the tumor cells of interest, and then kill those cancer cells.
  - B. The first two FDA approved CARs and the general course of patient care for CAR-T therapy
    1. Novartis received the first FDA approval in August 2017 for Relapsed/Refractory (R/R) Acute Myeloid Leukemia in pediatric patients.
    2. Kite/Gilead received the second FDA approval in October 2017 for R/R Large B-Cell Lymphoma in adult patients, including Medicare patients
    3. Typically, the cell collection occurs at the same hospital where the infusion is intended
      - a. These patients are quite sick as they are “relapsed/refractory” patients meaning prior therapies, up to and including stem cell transplant, have failed to produce lasting remission.
      - b. Cell collection is usually around 2-weeks prior to infusion and involves hospital staff to perform the cell collection and to package and label in a manner that protects PHI and handle the cells per the manufacturer process for safe transport to the manufacturing facility. This typically involves cell lab staff who are trained and usually two staff are involved so this is a resource intensive process.
        - (i) At times, the cell collection may occur at one hospital and then the infusion at a second hospital.

- (ii) The patient may be too sick to tolerate outpatient cell collection and it may occur inpatient.
- c. The genetic engineering process typically takes around 2-weeks after which the manufacturer ships the cells back to the infusing hospital. The cells are checked in by the cell lab staff and the reverse PHI labeling process occurs as well as safety checks on the cells and proper storage until the actual time of infusion.
  - (i) The manufacturing process may fail and either viable cells cannot be returned or the cells returned do not meet the FDA labeling requirement of viability.
    - (a) Each manufacturer has different methodologies if the clinician wishes to proceed and infuse cells that do not meet minimum FDA labeling requirements.
      - (1) Novartis calls this their Expanded Access Program and provides the cells for free
      - (2) Kite/Gilead requires the patient to be enrolled in a trial at one of their designated trial locations
  - (ii) Some patients die between the time of cell collection and completion of the engineering process.
- d. Prior to receiving the cells, the patients receive a mild form of ablation using chemotherapy agents to reduce the incidence of cancer cells and to better prepare for the CAR-T infusion.
  - (i) There is a higher incidence of pediatric patients receiving the infusion as outpatient.
  - (ii) Currently, there is a much higher incidence of adult patients receiving the infusion as inpatients.
- e. All patients get a reaction called a Cytokine Release Syndrome (CRS) which may “storm” to the point of requiring inpatient admission and/or significant additional drugs/services including the one FDA-approved CRS-treatment drug tocilizumab which is also expensive. Either or in addition to severe CRS, some patients may get a neurological CAR-T-cell-related encephalopathy syndrome or CRES.
  - (i) Close and frequent clinical monitoring is required by trained staff as responding to complications shortly upon presentation increases survival and is required.

- (ii) A baseline MRI for many patients is needed prior to CAR-T to enable better monitoring of neurological changes.
- (iii) When complications result in changed status, patients are often admitted as inpatients if they were not admitted for the infusion initially.
  - (a) Adult admissions are typically occurring within the 3-day payment window for PPS hospitals and definitely within the 1-day.
- (iv) A key sign of neurological changes has been described as the inability to use a cell phone or text.
- (v) An acronym for toxicity associated with CAR-T is CAR - TOX.

C. What are gene therapies?

1. Gene therapy is a way to fix a genetic problem at its source. The polymers are either translated into proteins, interfere with target gene expression, or possibly correct genetic mutations. The most common form uses DNA that encodes a functional, therapeutic gene to replace a mutated gene.
2. Sparks Therapeutics was approved for Luxturna, a gene therapy targeted at a hereditary gene that causes confirmed biallelic RPE65 mutation-associated retinal dystrophy and may cause complete vision loss or blindness in some patients in one or both eyes
3. This therapy may require one or more doses per eye to prevent blindness

D. Currently, the FDA-approved forms of cell therapies rely upon autologous cells (i.e., the patient's own cells), but there are allogeneic or donor cell products under development and gene therapies do not rely upon autologous cells.

1. Biologics in the pipeline and under Clinical Trial include allogeneic cell based products which may have "shelf life" and be universal as opposed to patient-specific with autologous products

E. Clinical Trials for Cell and Gene Therapies

1. Numerous clinical trials are underway including moving CARs from R/R to first- and second-line treatments and to move to solid tumors in addition to blood-based cancers
2. Furthermore, cell labs are able to genetically modify patient and donor cells in the laboratory, at bedside so to speak, using various "home brew"

using laboratory processes available to labs having complex lab CLIA certification.

- a. Investigational Review Board (IRB) approvals may be required
3. Significant investment is being made in these products which may extend beyond the logical economics of products aimed at orphan or small disease populations

### III. CMS' Clinical Trial Reimbursement Policy and Implications

A. CMS provides coverage for certain items and services for which coverage would not otherwise be available. This is especially important for drugs and devices not yet approved by FDA, which otherwise have no basis for coverage.

1. History of Clinical Trial Policy.
  - a. Initially implemented in September, 2000.
  - b. Proposed revisions published in April, 2007.
  - c. Modest revisions made on July 9, 2007.
  - d. New set of proposed revisions issued on July 19, 2007.
  - e. Proposal purportedly withdrawn on October 17, 2007.

B. CMS' Clinical Trial Policy covers various issues:

1. Studies subject to the policy must have certain desirable characteristics, such as the following:
  - a. Principle purpose is to test whether the intervention potentially improves the participants' health outcomes.
  - b. Trial is not unjustifiably duplicative of existing studies.
  - c. Trial is sponsored by a credible organization.
  - d. Trial complies with Federal regulations regarding the protection of human subjects.
2. Since it is difficult to determine whether a study meets these characteristics, CMS "deems" studies to have these characteristics if any of the following apply:
  - a. The trial is funded by NIH, CDC, AHRQ, CMS, DOD, or VA.

- (i) Includes trials supported by centers or groups funded by any of these agencies.
  - b. The trial is conducted under an IND reviewed by the FDA.
  - c. The trial is exempt from an IND.
- 3. Additionally, there are certain Medicare-specific requirements that must be met before the study is covered under the policy.
  - a. The study must involve the evaluation of an item or service that falls within a Medicare benefit category.
  - b. The study must have a “therapeutic intent.”
  - c. The study must enroll patients with diagnosed disease rather than healthy volunteers if therapeutic intervention. Diagnostic tests can have a control group.
- 4. Once a study is covered under the policy, there are certain costs that are covered, and other costs that are excluded:
  - a. Covered costs include:
    - (i) Items and services typically provided absent a clinical trial.
    - (ii) Items and services, such as administration of product, as well as certain services required solely for the investigation of the item, including appropriate monitoring of effects and prevention of complications.
    - (iii) Treatment of complications.
    - (iv) The investigational item itself, but only if it would otherwise be covered outside of the study.
  - b. Excluded costs include:
    - (i) The investigational item itself, if no coverage is available outside the study.
    - (ii) Data collection not directly related to clinical management.
    - (iii) Items and services customarily provided by research sponsors free of charge to any enrollee in the trial.

C. Applying CMS’ Clinical Trial Policy to CAR-T

1. One of the challenges in applying the CTP is that there is no standard of care (SOC) yet for CAR-T services – therefore, routine costs are hard to define and due to the extreme costs, there is incentive to argue that no services are SOC or routine
  - a. For example, autologous cell collection can be performed either in the hospital inpatient or outpatient setting
  - b. Furthermore, Novartis, the first FDA-approved manufacturer initially provided fair market value (FMV) contractual payment to hospitals for cell collection & lab processing services.
    - (i) Note that this is similar to Provenge and some speculate that CMS guided Novartis in this direction.
  - c. CAR-T Infusion often occurs inpatient, but may be outpatient, particularly for pediatric patients
    - (i) This raises questions such as whether full inpatient admission is the SOC for IV administration of CAR-T
    - (ii) Drugs to condition patient for cell collection & mild myeloablation prior to CAR-T are used based on FDA-labeled indications, but for CAR-T rather than stem cell transplants
    - (iii) Drugs for post-infusion complications such as Tocilizumab are FDA approved and are being used per label

#### IV. CAR-T Clinical Trial Compliance Issues

##### A. CMS CTP FAQs (MLN Matters SE0822)

1. Medicare payment is not available for any item or service used in a clinical trial where the sponsor agrees to pay for the costs of such item or service if a commercial payer denies payment.

##### B. OIG Pharmaceutical Manufacturer Compliance Program Guidance

1. The Anti-Kickback Statute is implicated when a manufacturer agrees to hold a customer harmless for non-payment, as that eliminates ordinary financial risk for the customer.

##### C. Beneficiary Inducement Special Bulletin (Aug. 2002)

1. OIG considers creating a safe harbor to protect value given as part of a clinical trial. However:

- a. OIG only considered this safe harbor protection for government sponsored trials.
- b. OIG never acted on this intention.
- c. Implication is that furnishing items at no charge, including waiver of coinsurance, for beneficiaries in clinical trials produces some risk, including, for example, furnishing combination drugs at no cost when used for their labeled indication.

V. Coverage

- A. The order of operations for insurance in the United States is eligibility, coverage and then payment; as a result, understanding CAR-T coverage by major payer group is imperative.
- B. Medicare fee-for-service has an informal practice that once a drug or biological is approved, then it is SOC and covered under either Part A or Part B.
  - 1. Cancer drugs have additional statutory requirements that guarantee liberal coverage for certain off-label uses
  - 2. Note that mid-2018, a major Medicare Advantage (MA) plan – United Healthcare-requested a National Coverage Analysis (NCA) of CAR-T. Note that MA plans have to cover and pay for FDA approved new drugs under their usual per member per month rates (PMPM), however, when a new NCA is announced, MA plans can avoid the cost for the service under the NCD generated from the NCA and CMS pays outside the usual PMPM until the MA contracted rates can “catch up” to include the cost of the new NCD-covered item or service. It is presumed then that United Healthcare initiated the NCA to avoid cost of CAR-T – an unusual process for a drug or biologic, but not unexpected at this price point.
  - 3. The draft NCD for CAR-T is expected mid-February 2019 with a typical 30-day comment period
    - a. The final NCD is expected around May 2019 and may be finalized as a full NCD or possibly a Coverage with Evidence Development policy or CED, including requiring a registry of all Medicare CAR-T patients.
- C. Medicaid coverage is state-by-state
  - 1. Some states are covering and payment includes recognition of the cost of the cells including New York.
  - 2. Other states refuse to cover or pay for CAR-T such as Illinois.

D. Commercial:

1. The Blues Distinction Centers for Cellular Immunotherapy is tasked with developing coverage and payment policies for CAR-T and is expected to be the guideline for most Blue Cross/Blue Shield plans in the US and may be adopted by other national payers

VI. Commercial Product Reimbursement – Medicare

- A. Once FDA approves a drug or biological that requires qualified clinical staff administration (i.e., as opposed to being a non-covered self-administered outpatient drug), the drug usually is covered by CMS whether by Part A or Part B, particularly antineoplastic drugs or drugs for cancer treatment

1. Part A Payment under the Inpatient Prospective Payment System (IPPS):

- a. The current short-term acute care hospital payment methodology is Medicare Severity-Diagnosis Related Groups or (MS-DRGs) which rely on a statutory and well-established historical rate-setting methodology

- (i) The methodology uses hospital inpatient claims from two-years prior to the federal fiscal year (e.g., 2017 claims are used for 2019 payment rates)

- (ii) Hospital cost reports from about three years prior to the applicable rate year are inflated to current period and used for rate setting

- b. Due to this history-based method, payment rates obviously exclude new technology approved subsequent to these timeframes which led Congress to approve the New Technology Add-On Payment (NTAP)

- c. CMS typically does not establish new MS-DRGs for new clinical services despite having statutory authority to do so

- (i) The last known example of CMS establishing new DRGs to address a new technology was 2003 when CMS created brand new DRGs for a new cardiac device called drug-eluting stents or DES

- (ii) CMS has not invoked this authority since

2. Despite significant costs of new technology, Medicare beneficiaries pay one deductible per Part A benefit period of \$1,364 for 2019 & coinsurance of 25% of this amount for each day 61-90 of a benefit period

3. Part A payment for PPS-exempt cancer centers
  - a. The eleven PPS-exempt cancer centers receive a payment based on case rate following pre-IPPS payment under TEFRA
  - b. Since CAR-T is a treatment for cancer, a very high proportion of CAR-T commercial and clinical trial cases are performed by the PPS-exempt centers.
  - c. The TEFRA payment rate is based on historical base cost which is not updated frequently (e.g., about every 12-15 years), but does have an exception process which is also labor intensive for the centers with strict criteria and also takes a significant amount of time to achieve. TEFRA-based payment does not have a provision for real-time outliers or new technology as is the case under IPPS, thus, there is no short term relief for CAR-T costs at the PPS-exempt cancer centers.
  
- B. When faced with extraordinary cost of CAR-T, CMS was heavily lobbied for financial relief for CAR-T
  1. The extreme high cost of CAR-T at an approximate \$400,000 price point was and is not recognized in any single MS-DRG payment rate
  2. Furthermore, there are a limited number of hospitals with the capability to provide CAR-T and several of the qualified hospitals are the eleven PPS-exempt cancer hospitals which are paid for Part A under a TEFRA rate and are not eligible for outliers or NTAPs as are PPS hospitals
  3. Poor Medicare payment is likely to limit access for Medicare beneficiaries; but CMS says they rarely, if ever, actually “see” restricted patient access happen even though this risk is often invoked in advocacy
  
- C. IPPS-considerations
  1. MS-DRGs must be budget neutral and extreme high cost will divert \$ from other ubiquitous cases to CAR-T cases
  2. NTAPs do not have to be budget neutral
  3. Impact on outlier payments; underpaying CAR-T can result in more outliers and impact on future outlier formula
  4. CAR-T cost is a non-labor cost and if built into MS-DRG would be subject to wage index & DSH & IME adjustments

- D. IPPS payment rates are comprised of national standardized amounts adjusted for local geographic wage and hospital-specific adjustments for teaching status and disproportionate share
1. For federal fiscal year 2019, CMS made the decision to group inpatient CAR-T cases based on the presence of one of two ICD-10-PCS codes for the administration of CAR-T products to MS-DRG 016 for autologous stem cell transplants as clinically, this is the most similar existing MS-DRG to CAR-T. <83 FR 41172>
  2. Both Novartis and Kite/Gilead submitted New Technology Add-On Payment (NTAP) applications to CMS and per the final Inpatient Prospective Payment System 2019 Final Rule, both products met the criteria for NTAP <83 FR 41283>
    - a. By regulation, NTAP payments are not to exceed the lesser of 50% of the cost of the new technology or 50% of the amount that the calculated cost of the inpatient case exceeds the MS-DRG payment <42 CFR 412.88(a)(2)>
    - b. Note that this formula relies on the hospital's billed charges and the mark-up applied to the expense-based new technology when included as a charge on the hospital's inpatient bill.
  3. Outlier payment is also available and the calculated cost of each MS-DRG case determined by CMS by applying the hospital's overall cost-to-charge ratio (CCR) determined from its most recent cost report to the individual patient's case covered billed charges is compared to the MS-DRG payment plus a fixed outlier threshold. If NTAP is applicable, which it is for CAR-T, then the NTAP payment is also added to the MS-DRG payment and the fixed outlier threshold, if any calculated cost exceeds these amounts, then the hospital receives 80% as additional outlier payment. <42 CFR 412.84>
  4. The key takeaway is that hospitals must mark-up their expense items such as CAR-T at a cost of \$373,000 by their CCR in order to achieve no more than 50% payment of the actual out-of-pocket cost for the new technology. For CAR-T, this means a cost of \$373,000 for an average hospital with an overall CCR of 0.25 must bill a gross charge of \$1,492,000 on the inpatient claim to the Medicare Administrative Contractor or MAC.
- E. Medicare outpatient hospital payment under the outpatient prospective payment system or (OPPS)
1. The first FDA-approved product was targeted to the pediatric population and with this product and patient population, the infusion often occurs as outpatient hospital.

2. Based on the outpatient setting and the fact that the products rely on autologous cell collection, CMS considered Provenge a corollary to CAR-T and established HCPCS product codes that include the leukapheresis and dose preparation procedures.
  - a. Provenge (HCPCS code Q4043) approved in 2011 is a very poor example as it is not a common service and the cell collection is most often performed in separate cell collection facilities that are not hospitals or in physician offices
  - b. Initially, Novartis contracted with and paid hospitals a negotiated fair market value (FMV) for the cell collection and dose preparation procedures but based on widespread feedback from hospitals and professional associations, this practice ended as of January 1, 2019 when the AMA CPT codes for these services became effective.
  - c. CAR-T products are biologics with national drug codes (NDCs) and manufacturer reported average sales prices (ASPs)
    - (i) As new drugs, CAR-Ts are paid ASP +6% under OPSS. Refer to the most current Addendum B for the applicable payment rates. <https://www.cms.gov/Medicare/Medicare-Fee-for-Service-Payment/HospitalOutpatientPPS/Addendum-A-and-Addendum-B-Updates.html>
    - (ii) As of January 1, 2019, Kymriah (Q2042) has a higher payment rate than Yescarta (Q2040) due to the averaging of the pediatric and adult indication prices of \$475,000 & \$373,000, respectively
    - (iii) Initially, industry and patient advocates were very concerned with the high co-payment amounts published in Addendum B showing 20% of the allowable payment amount. However, no single Medicare OPSS co-payment amount can exceed the applicable annual inpatient deductible which is \$1,364 for 2019. The patient pays this amount and CMS pays the remainder of the applicable payment amount. <42 C.F.R. 419.41(c)(4)(i)>
      - (a) This likely led CMS to add the indicator to Addendum B in 2019 that the published co-payment will not exceed the deductible.
3. There were no AMA CPT codes for CAR-T services until January 2019 when AMA defined new Category III codes (0537T-0540T)

- a. Despite comments from hospitals and the AMA CPT codes describing discrete CAR-T services, CMS retained hospital cell collection and lab dose preparation and processing services in the long descriptions of the two CAR-T product Q codes (Q2040 and Q2042) in 2019
  - (i) CMS agreed in the 2019 OPSS Final Rule that the infusion of CAR-T should not be reported with chemotherapy or SCT transplant CPT code and established a separate payment rate for the outpatient infusion of CAR-T (0540T).
  - (ii) CMS did not recognize the other 3 AMA CAR-T CPT codes (0537T-0539T) and instead assigned status “B” which colloquially means a “better/different code exists” presumably the Q code for the product
  - (iii) Per Administrative Simplification Act requirements, HCPCS codes are only applicable on outpatient claims
  
- 4. The National Uniform Billing Committee (NUBC) established new claims transaction set codes for hospital reporting of CAR-T services effective April 1, 2019
  - a. CMS and other payers and hospital providers must comply with Administrative Simplification Act claim and code set transactions <https://www.cms.gov/Regulations-and-Guidance/Administrative-Simplification/HIPAA-ACA/index.html>
  - b. CMS acknowledged that providers should report CAR-T services on hospital claims using the newly designated NUBC codes including the new value code to report the acquisition cost of the product on claims
    - (i) “...for the reasons stated previously, there is no separate payment by Medicare for these steps in the manufacturing process. However, it will be possible for Medicare to track utilization and cost data from hospitals reporting these services, even for codes reported for services in which no separate payment is made. The CAR T-cell related revenue codes and value code established by the NUBC will be reportable on HOPD claims, and will be available for tracking utilization and cost data, effective for claims received on or after April 1, 2019.” <83 FR 58406>

F. Medicare payment under the Medicare Physician Fee Schedule (MPFS)

- 1. CPT codes 0537T through 0539T for the cell collection and lab processing services have a status code “B” under the MPFS which means “bundled.”

- a. If the physician performs the cell collection (0539T) then presumably there is another professional service performed on the same date that will be billed such as an evaluation and management service (CPT codes 99211-99215)
2. CPT code 0540T has a status code “C” for carrier priced, so physicians will have to submit a letter to their MAC when they perform this service and bill.

## VII. Commercial Product Reimbursement – Medicaid

- A. Novartis & CMS made a “big splash” of outcomes-based payment when FDA approved the pediatric indications product. Outcomes were defined as “complete response” within 30 days of infusion. <https://www.cms.gov/newsroom/press-releases/cms-innovative-treatments-call-innovative-payment-models-and-arrangements>
  1. Politico reported in July that CMS officials quietly cancelled the plan after it drew internal and external scrutiny & criticism
- B. Medicaid programs are unprepared for extreme high cost so payment ranges from cost-based “carve out” for product (NY) to no separate product payment (Managed Medicaid in KS) to no coverage for product at all (IL)
- C. Medicaid programs typically do not have NTAP and usually a less generous outlier formula, if at all

## VIII. Commercial Product Reimbursement – Commercial Insurance

- A. Once coverage is confirmed with a payer, the payment amount is negotiable and the hospital should prepare and execute single case agreements (SCA) with each applicable payer.
  1. Many hospitals that are certified to perform CAR-T services perform stem cell transplants (SCTs) and their commercial payer are critical to commercial payor coverage and payment
  2. The SCT contracts are often used as the initial “template” for the CAR-T single case agreements.
  3. Most attempt to ensure payment at no less than invoice cost of the CAR-T product is “carved-out” in addition to having a stop-loss provision for high patient care services
  4. Additional services for CAR-T patients such as baseline MRI for neurological status & carve-out for other high cost drugs like tocilizumab

5. Peer-to-peer prior authorization meaning the patient's CAR-T physician must discuss the case with the payer's medical director, is common. It is important to outline the details around this communication including timeframes to shorten length of time required as delays in patient care have life and death consequences.
- B. Education/advocacy with payor medical directors or medical policy coordinators to add/clarify/expand coverage may also be needed.

IX. Compliance Considerations relating to Commercial Product

- A. Relationships with manufacturers often raise questions about whether the Anti-Kickback Statute (1128B of the Social Security Act) is implicated. The AKS attaches criminal and civil monetary penalty liabilities to knowingly and willfully furnishing anything of value in exchange for referring or recommending an item or service reimbursable under Medicare or Medicaid.
- B. Examples of the types of arrangements that raise the specter of the AKS (but don't necessarily violate it) include:
  1. A manufacturer overpaying for services from a provider that is also a purchaser of its products
  2. A manufacturer providing compensation to a provider customer to hold it to avoid reimbursement shortfalls.
  3. A manufacturer offering to pick up the copay for Federal healthcare program beneficiaries.
- C. Protection for some arrangements, such as payment for T-cell collection, can be found in the personal services safe harbor, which requires:
  1. A signed contract between the parties for a term of a least a year.
  2. A full description of the services being purchased.
  3. Payment at fair market value that does not take into account the value or volume of any business generated.
  4. The services purchased are the minimum services needed.
- D. Pharmaceutical Manufacturer Copayment Coupons Special Advisory Bulletin (Sept. 2014)
  1. OIG found that manufacturers had insufficient controls to prevent Federal healthcare program beneficiaries from using copay support programs

2.     OIG stated that use by Federal healthcare program beneficiaries was a violation of the AKS
3.     To be safe, hospitals can therefore only seek support for patient-liable portions of gene therapy if the patients are commercial pay patients