Vineti

Chain of Identify (COI) Identifier

An industry standard recommendation for personalized therapeutics

As presented to the Standards Coordinating Body for Regenerative Medicine, October 2020

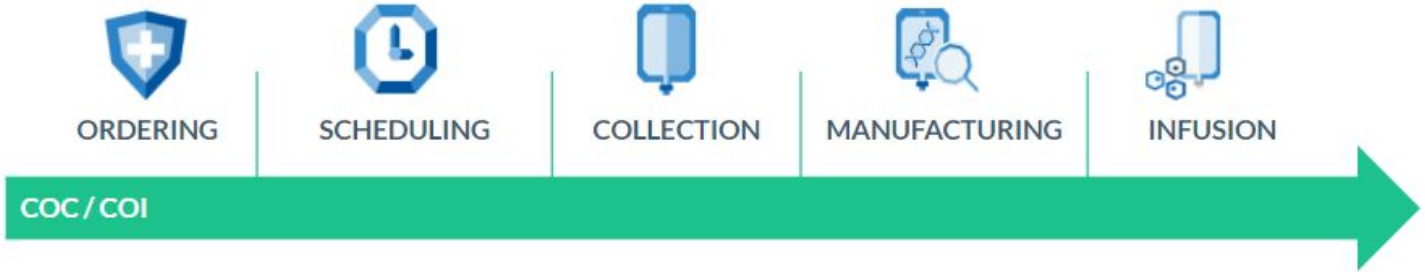
Background: Chain of Identity and patient identifiers

Because personalized therapeutics must be traced and matched to the intended patient

In personalized therapeutics, such as CAR-T cell therapies, safety and efficacy rely on the right patient receiving the right treatment.

This process of tracking and matching is driven by a core concept known as **Chain of Identity (COI)**, or the permanent, transparent association of a patient and/or donor's unique identifiers to their tissue and/or cells from order through collection, manufacturing, administration, and post-treatment monitoring. Pairing COI with **Chain of Custody (COC)** -- or the permanent data capture of tracking and handling information for a therapy at every step -- provides a truly traceable, trustworthy process.

At the most basic level, Chain of Identity is enabled by a **COI Identifier (COI ID)**, a set of numbers and/or characters that is the overarching "ID badge" for each patient and key materials related to their product. The COI ID must link a therapy to the intended patient *and* protect patient privacy. The COI ID is used in multiple places on the patient journey, from medical records to in-process drug labels.



Background: the need for a new approach to COI ID

Because personalized therapeutics must be traced and matched to the intended patient

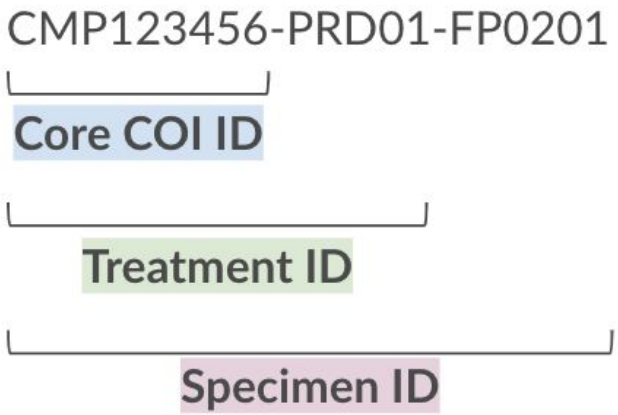
The current approach to the structure and format for COI IDs is based on existing patient-specific, donation-based processes, such as bone marrow collection.

While these prior approaches to COI ID are well-known, they are not sufficient for cell and gene therapies (CGTs). CGTs are often more complex than other donation-based treatments. Some treatments may be patient-based, some may be donor-based, and some may be based on a mix of source materials. Some may be single-dose products, while others may be multi-dose products.

Based on extensive experience, Vineti believes that the advanced therapies industry needs an updated standard for COI ID.

The Standards Coordinating Body for Regenerative Medicine, an independent non-profit organization working to drive industry standards, has a current workstream on cell therapy labeling standards. Vineti presented the following vision for COI ID to the SCB working group as part of an ongoing standards discussion in October 2020.

Vineti's recommended COI ID approach



Vineti Presentation to SCB

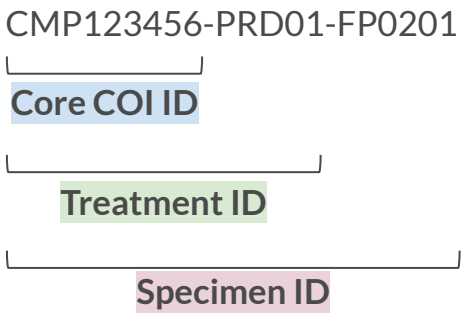
COI ID standardization - introduction

- The purpose of this recommendation is to highlight the complex problems associated with Chain of Identity and to provide a solution to each use case scenario.
- This recommendation was informed by a variety of sources, including guidance and prior interaction with the FDA and our own real world insights with patient data.
- The elegance of this solution is in its simplicity and its scalability - we will continue to evolve as the needs of this market change over time. Meanwhile, we have a strong opinion about how best to meet the needs of this market today, including the needs of healthcare providers.
- COI is the backbone to any platform of record and each unit of the ID was chosen with a deliberate function in mind. There is nothing extraneous and each data point is intentional.
- Our goal is to describe a single ID that best meets the data needs of the patient, the site and the product Company through a range of use case examples: auto, allo and PCV.

Why we need a COI Standards Recommendation

- Why COI matters - when workflow changes occur
  - Changes to treatment, changes to sample type, an order restart for any reason
  - Importance of flexibility with accuracy
- Why this data is so valuable
  - To Patients, to the HCPs and to Pharma companies for patient care, patient safety and workflow improvement
- Requirement for all companies with an IND or BLA is an annual report
  - Events such as procedure failure or multiple products must be explained to the FDA
  - With COI the events of each lot are clearly visible, especially important if you've had to go back to patients with invasive procedures
- The root identifier informs all integration opportunities -- such as EHR
- The root identifier enables patient safety and patient care

Vineti's proposed COI ID structure



Core COI ID

Uniquely identifies the patient receiving the therapy and the company/sponsor for the therapy product

Treatment ID

Uniquely identifies a specific treatment order cycle for a therapy; distinct part of treatment journey

Specimen ID

Uniquely identifies each specimen involved in treatment, including cellular material, and any manipulation of that material, through to the final product

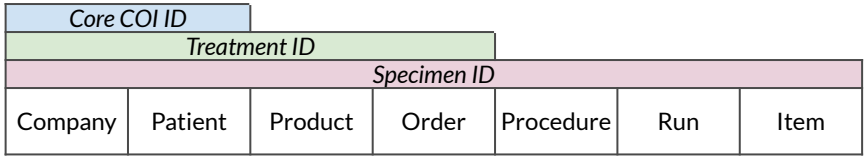
Vineti's proposal for COI ID

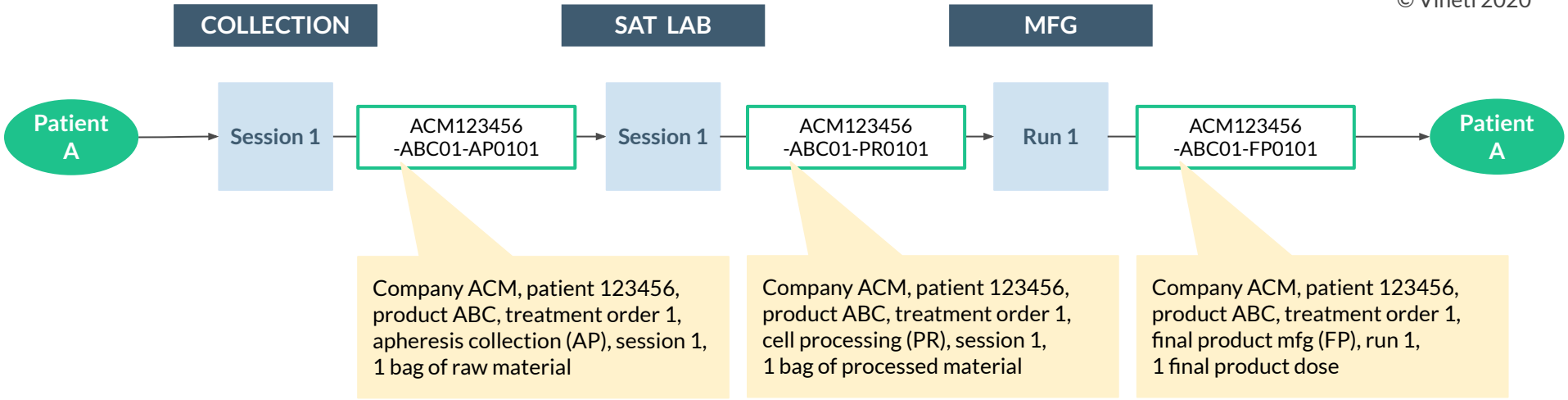
Core COI ID						
Treatment ID						
Specimen ID						
Company identifier	**Core Patient ID**	**Product identifier**	**Order identifier**	**Procedure identifier**	**Run identifier**	**Item identifier**
Unique to a company/sponsor	Unique to a patient for all their product journeys	Unique to a product (or therapy)	Indicates a specific treatment order cycle	Indicates a procedure type and/or grouping of specimens	Indicates a specific session/run of a procedure type	Indicates item number produced in a session/run

- Accommodates wide variety of therapy products and simple/complex treatment journeys, a flexible standard
- Uniquely identifies therapy and therapy recipient, different treatment cycles, and each specimen (materials, doses) involved
- Developed with simplicity and predictability for HCPs in mind

Autologous therapy 1

Single collection, single dose

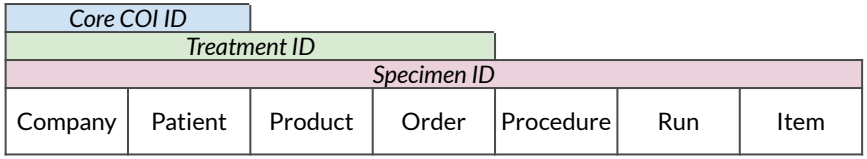


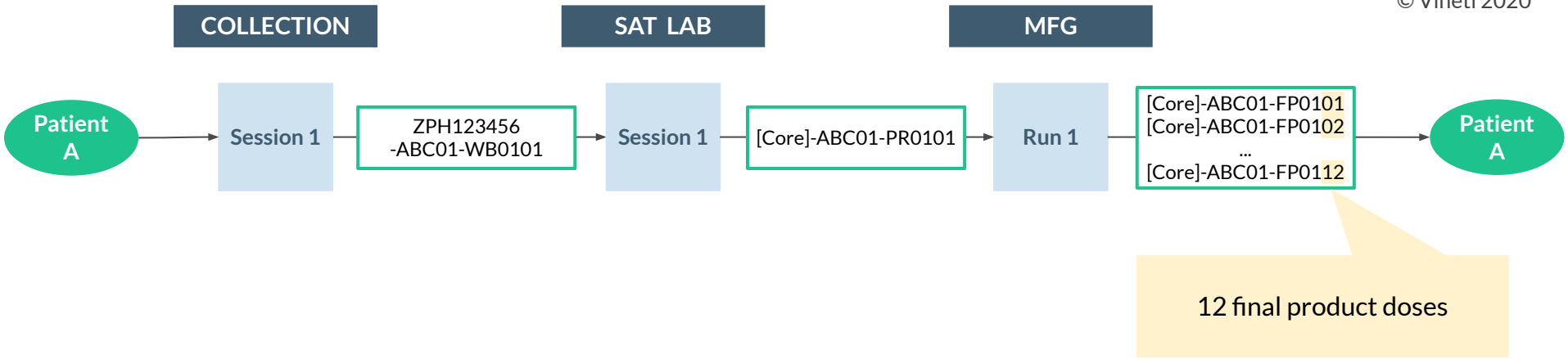


Suzy Smith, with a rare genetic disorder, will receive Acme Pharma's product ABC and requires 1 mobilized apheresis. Only 1 aph bag was collected to get the required dose. The dose is manufactured successfully.

Autologous therapy 2

Single collection, multiple doses



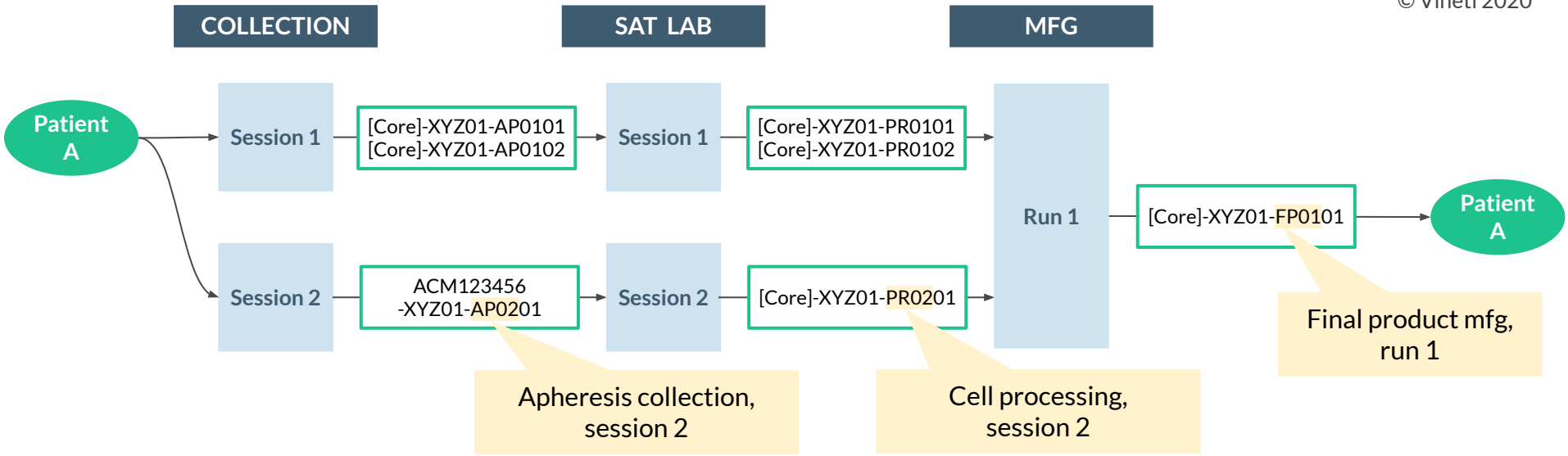


Bob Olson has cancer and will receive Zephyr Pharma's EBV T-cell product (ABC), in phase 3 clinical trial. He provides a whole blood unit. 12 doses are created from that blood unit.
All final product doses were manufactured successfully.

Autologous therapy 3

Multiple collections, merged manufacturing run

Core COI ID						
Treatment ID						
Specimen ID						
Company	Patient	Product	Order	Procedure	Run	Item

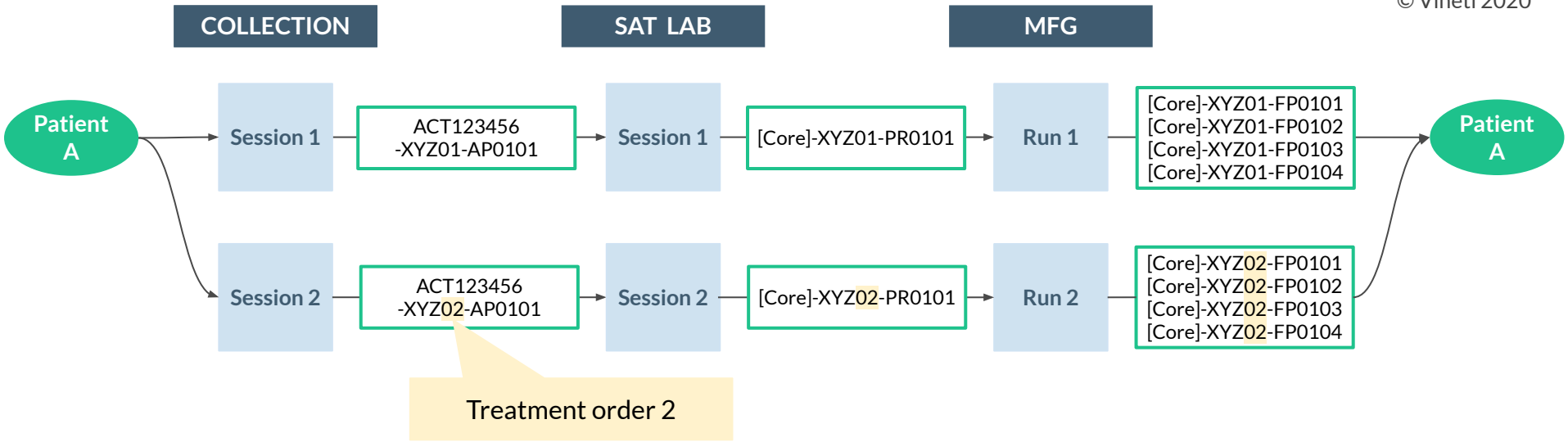


Jane O'Brien has a rare disease and will receive Acme Pharma's product XYZ, which requires 2 mobilized aphereses to get the required dose combined into one finished product. The first apheresis created 2 bags, and the second created 1 bag. All doses mfg successfully.

Autologous therapy 4

Multiple treatment orders, multiple doses

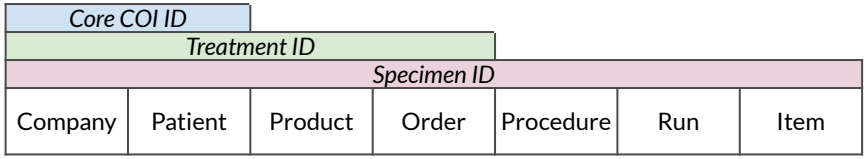
Core COI ID						
Treatment ID						
Specimen ID						
Company	Patient	Product	Order	Procedure	Run	Item

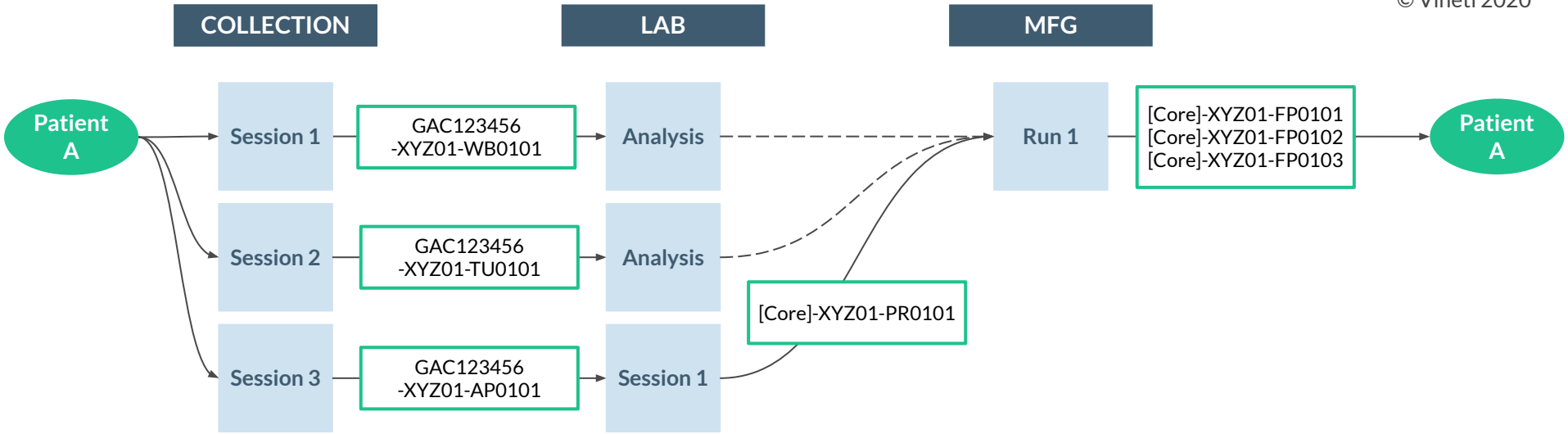


Fred Smith, with an autoimmune disease, is enrolled in Acme Cell Therapy's clinical trial using T-reg therapy (XYZ) that will have 2 aphereses performed 4 weeks apart. Each apheresis will produce 4 frozen doses. All doses mfg successfully.

PCV

Personalized Cancer Vaccine / autologous cell therapy

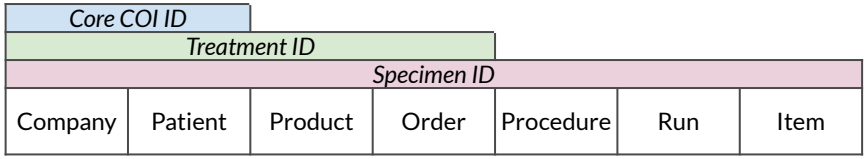


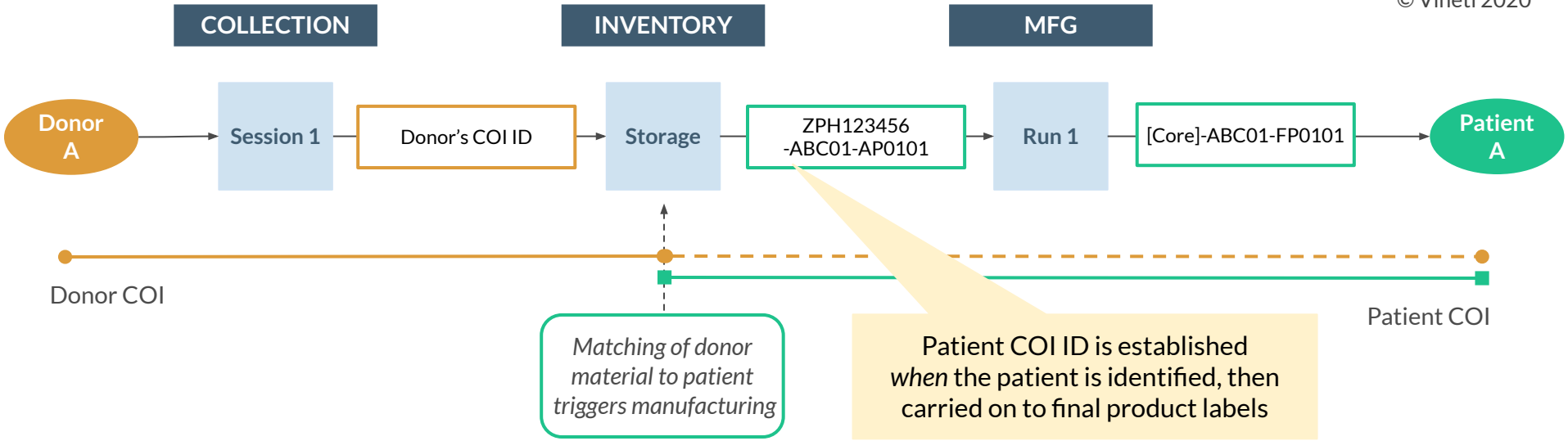


Digger Carlson has pancreatic cancer and enrolls in Gene Alpha Cells' phase 2 trial with a personalized gene modified T-cell product (XYZ). Digger provides a whole blood sample, a tumor excision, and an apheresis to make 3 final product doses from one bulk drug product. All parts of the process are manufactured successfully.

Allogeneic therapy

Manufacture from inventory, donor bank





Abraham Scott has leukemia and enrolls in Zephyr Pharma's clinical trial using a neutrophil engraftment therapy from donor cord blood (ABC). A donor is matched through a cord blood bank, and the donor's frozen cord blood is sent to manufacturing to produce 1 final product dose.

Vineti

Thank you!

Vineti Chief Strategy Officer Louise Pacini and Product Manager Amy Fonte presented this COI ID proposal to the Standards Coordinating Body labeling working group in October 2020.
For more information, please contact info@vineti.com.