

New Products and Processes to Address Patient Needs

Reflecting on the Future

Prof. Charles L. Cooney

Department of Chemical Engineering

MIT Cambridge, MA USA

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Required Disclaimer

I can't predict the future but this is how I think about it

2030 is close enough that we can guess what will happen but far enough that we really don't know

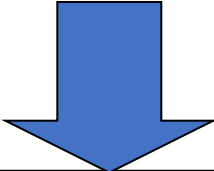
What are the Principles on which I think about the future?

An Industry Under Stress

Where are we today?

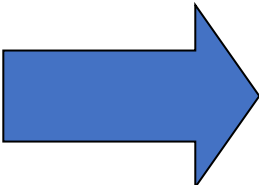
Where do we want to go?

Health Care & Pricing Pressure

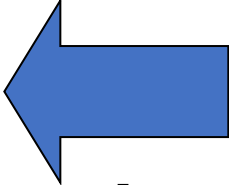


BIOTECHNOLOGY & PHARMACEUTICAL INDUSTRY

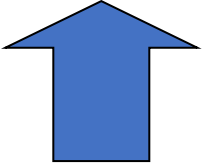
Patent Expiration



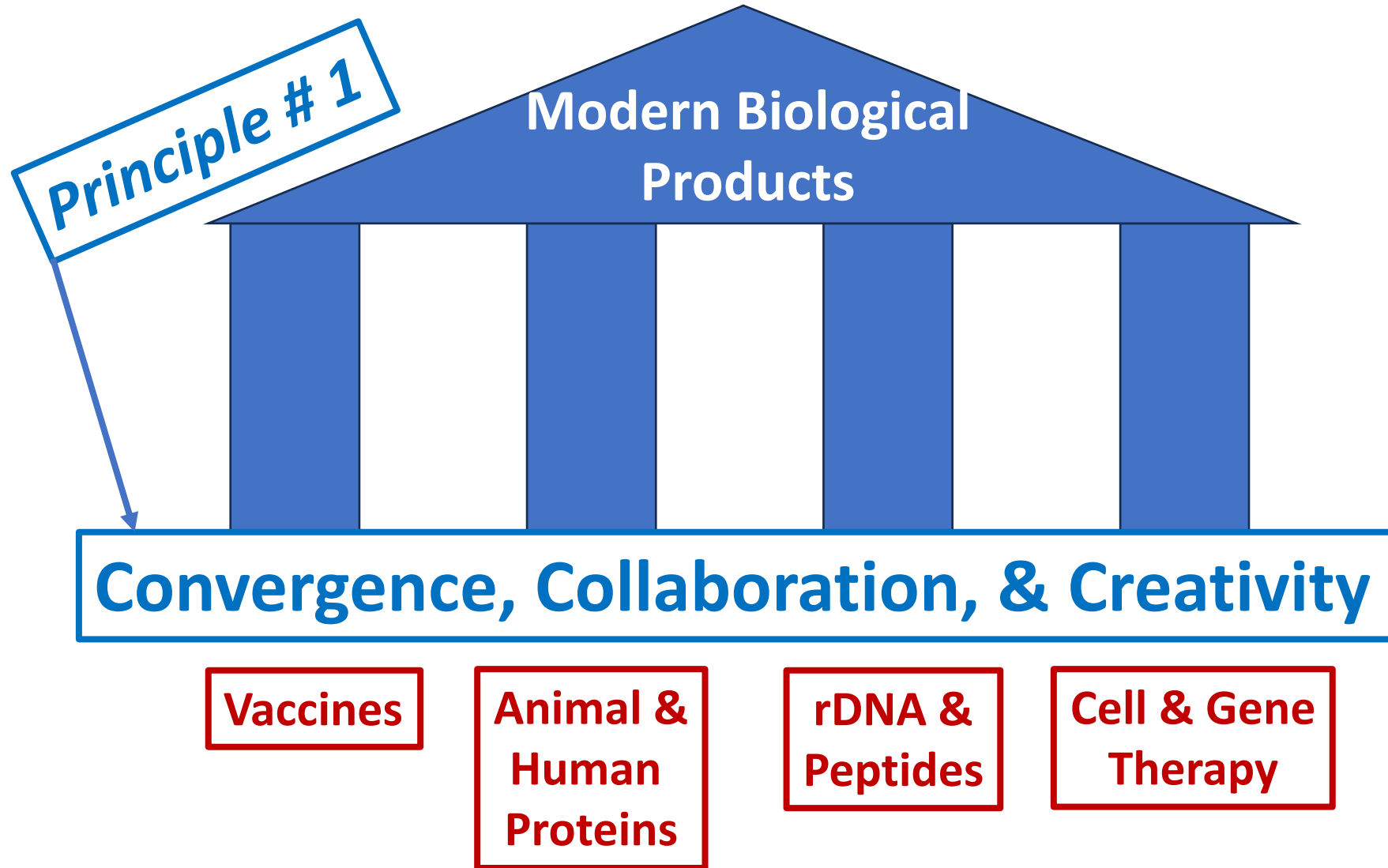
Low R&D Productivity



Specialty & Precision Medicine
Patient Access



Reflecting on the History of the Future

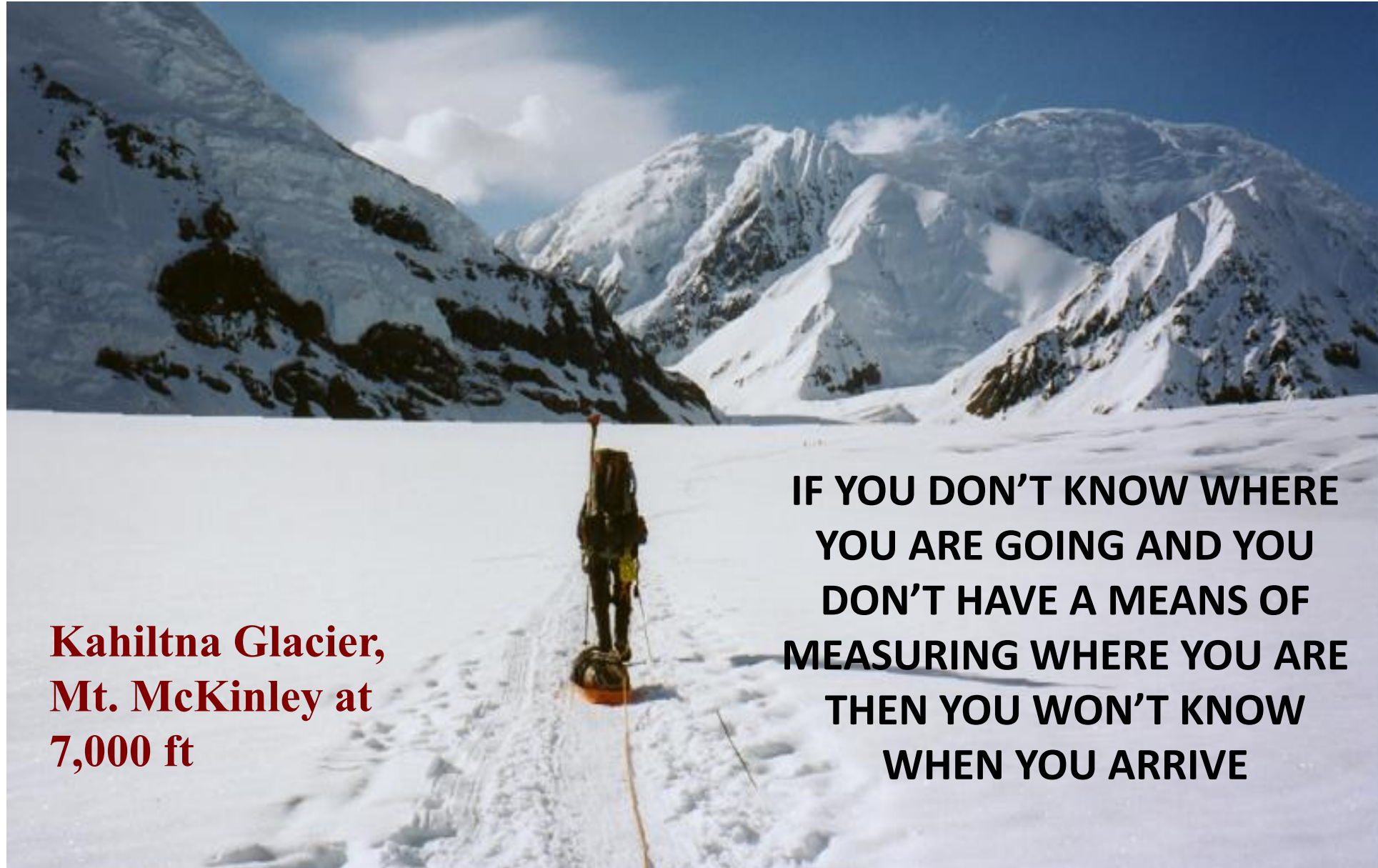


Top of Mind Trends

- **Accelerated approvals** puts manufacturing on the critical path
- **New Modalities** evolving as vehicles for Delivery – blurring DS & DP
- **Product potency & Precision medicine** require innovation in manufacturing
- **Product and process analytics** enable product by process – critical to ATMPs
- **Data as an asset**, connecting science to the patient requires a digital strategy

Envisioning the Future

Principle #2

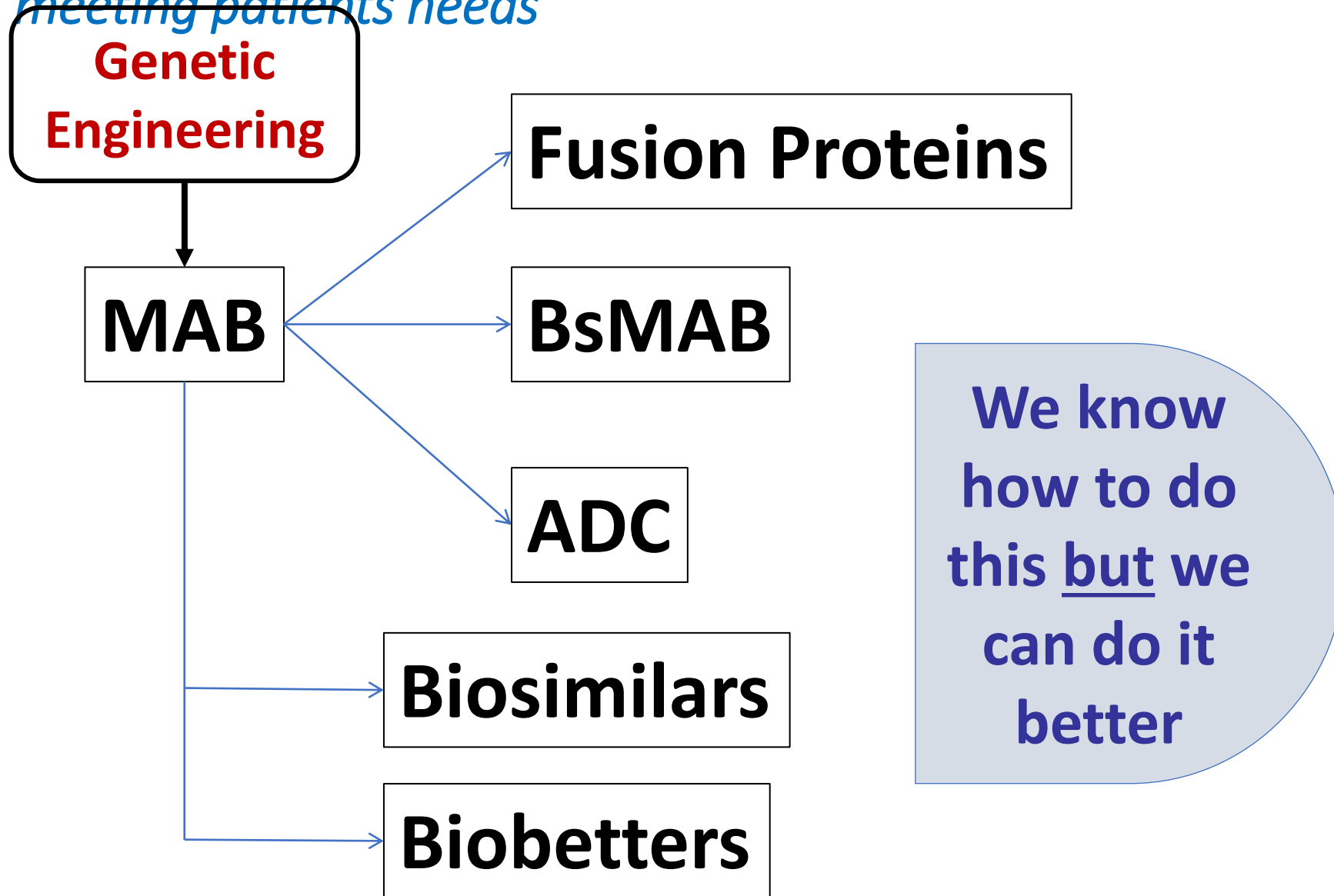


**Kahiltna Glacier,
Mt. McKinley at
7,000 ft**

**IF YOU DON'T KNOW WHERE
YOU ARE GOING AND YOU
DON'T HAVE A MEANS OF
MEASURING WHERE YOU ARE
THEN YOU WON'T KNOW
WHEN YOU ARRIVE**

40 Years Building Product and Process Platforms

meeting patients needs



We Now Need to Build New Platforms

New Modalities (ATMPs) Require Fit-for-Purpose Technology

- Gene Therapy
 - **Replace** protein replacement therapy
 - **Repair** thru gene knockdown and regulation
 - **Edit** thru CRISPR, etc.
- Immune regulation
- RNA therapeutics (sRNAi, mRNA)
- Cell Therapy
- Microbiome

**Do we know
how to do
this?**

Embrace Complexity & let Biology do the Work

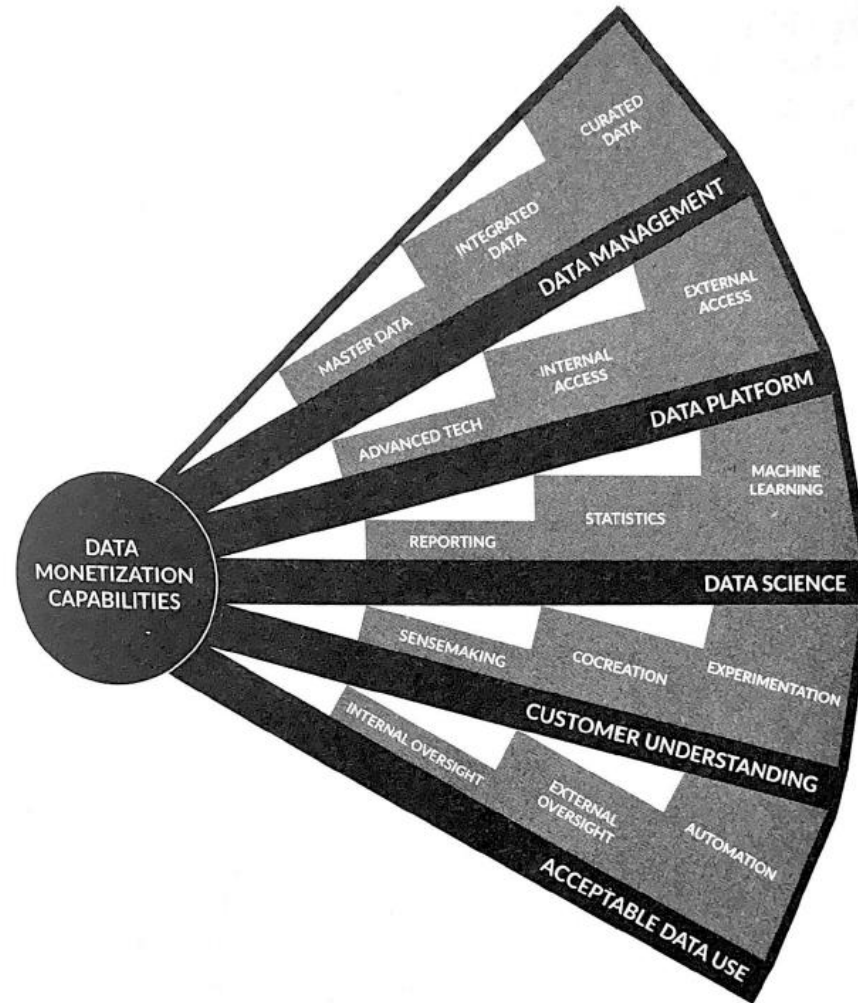
What is the thread that weaves biological products into a fabric of health care?



Principle # 3 Threading the needle with DATA

- Biological products are defined by a process
- **We define the product, its attributes, and the process with rigorous analytics**
- **The analytics provide us with data**
- **The data & models provide insight**
- **Insight drives decisions and action**

A Framework for Value Creation with Data Monetization Capabilities*



Value Creation with Data

- *Data Collection*
- *Analytics & Insight*
- *Action*

* *“Data is Everybody’s Business”* by B. Wixom, et al. (2023) MIT Press

Lessons Learned from Data, Models & their Application

- **WHY** –
 - Understanding relationships between dependent and independent variables
 - Facilitating design and scale-up
 - Operation and Control
 - Compliance, process verification
 - Framework for sharing data
 - Visualization
 - Economic Analysis
- **WHAT** – Creation of value with data
- **HOW** – Data Collection, Analytic Insight, & Action

Principle # 4

Framework for decisions in bioprocess development – *creation of value*

- **Speed** of rate of rate controlling step
 - Time to market
 - Operation
- **Quality** improvement and compliance
- **Cost** of project & COGS
- **Flexibility** in schedule, facilities use, alternatives
- **Sustainability** reducing waste, water, & energy

Clarity in your GOAL as you embrace & manage risk along each step of the journey

Principle #5

Approaching Ama
Dablam Summit
at 7,000 m

The GOAL of pharmaceutical manufacturing is the sustained delivery of a quality (safe and efficacious) product to the patient

**Delivery to the
Patient**

**Sustain the
Business**

**When you reach the summit
you are 25% towards your goal**

