ex events New Product Planning SUMMIT

Making the Target Product Profile a Tool

and Not a Task to be Completed

Brad Payne, Partner,
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ARTISAN Healthcare Consulting

Facts. Insights. Actions.

About Me

Brad Payne, Vice President, Artisan Healthcare Consulting

PARTNER at Artisan Healthcare Consulting, focused on New Product Planning & Development

FOUNDER of "BioPharma New Product Development, Marketing and Innovation Strategies"

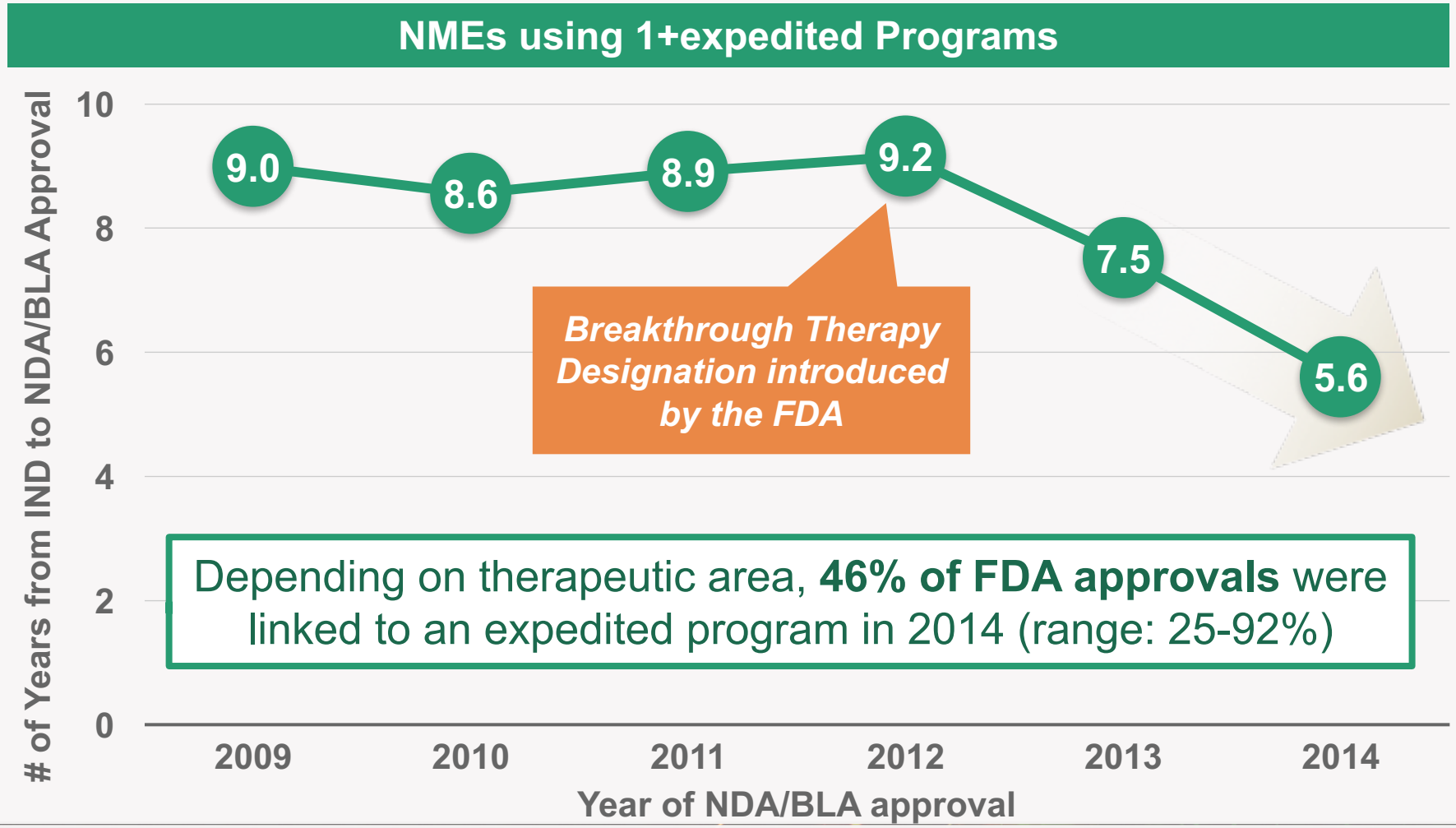
Join on LinkedIn!

WRITER – published 5 white papers over the past 18 months

Making the Target Product Profile a Tool
and Not a Task to be Completed

1	**Is a TPP important?**
2	Define what the TPP is and what it is not
3	Use the TPP to increase engagement and alignment with key stakeholders
4	Determine the right data needed to inform TPP discussions
5	Understand how a TPP should evolve over time

With the introduction of breakthrough therapy designation, the approval process is being shortened



Source: Artisan analysis of FDA approved products, January 2009 - August 2014. Expedited pathways include breakthrough designation, fast track designation, accelerated approval, and priority review

Drug development challenges are increasing…

GILEAD
Advancing Therapeutics. Improving Lives.

Competitive entrants in the Hepatitis C area, leading to massive rebates

NOVARTIS

Rare disease franchise accused of ”gouging” patients with new CAR-T therapy pricing

AMGEN®

Repatha® has faced significant insurance coverage issues

...Oncology is one example

	Traditional Targeted Therapy	Novel IO Therapy
Indications	**1 INDICATION** 2L+, ER+ metastatic BC **Total Trial Arms: 5**	**3 INDICATIONS** 2L+ metastatic CRC, NSCLC, TNBC **Total Trial Arms:** 12
Trial Size	**152 patients**	**432 patients**
Trial Timing	**3.5 years** April 2016 to October 2019	**2.5 years** August 2016 to January 2019

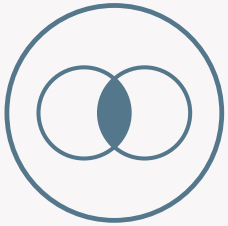
Trials have dramatically increased in scope and size, requiring NPP to commit more resources to create comprehensive market assessments of multiple indications and portfolio assets

How can a TPP help in this environment?

Shorten Development Timelines

Quickly elevate key issues

Drive cross-functional alignment on product attributes



clinical differentiation

strong value proposition

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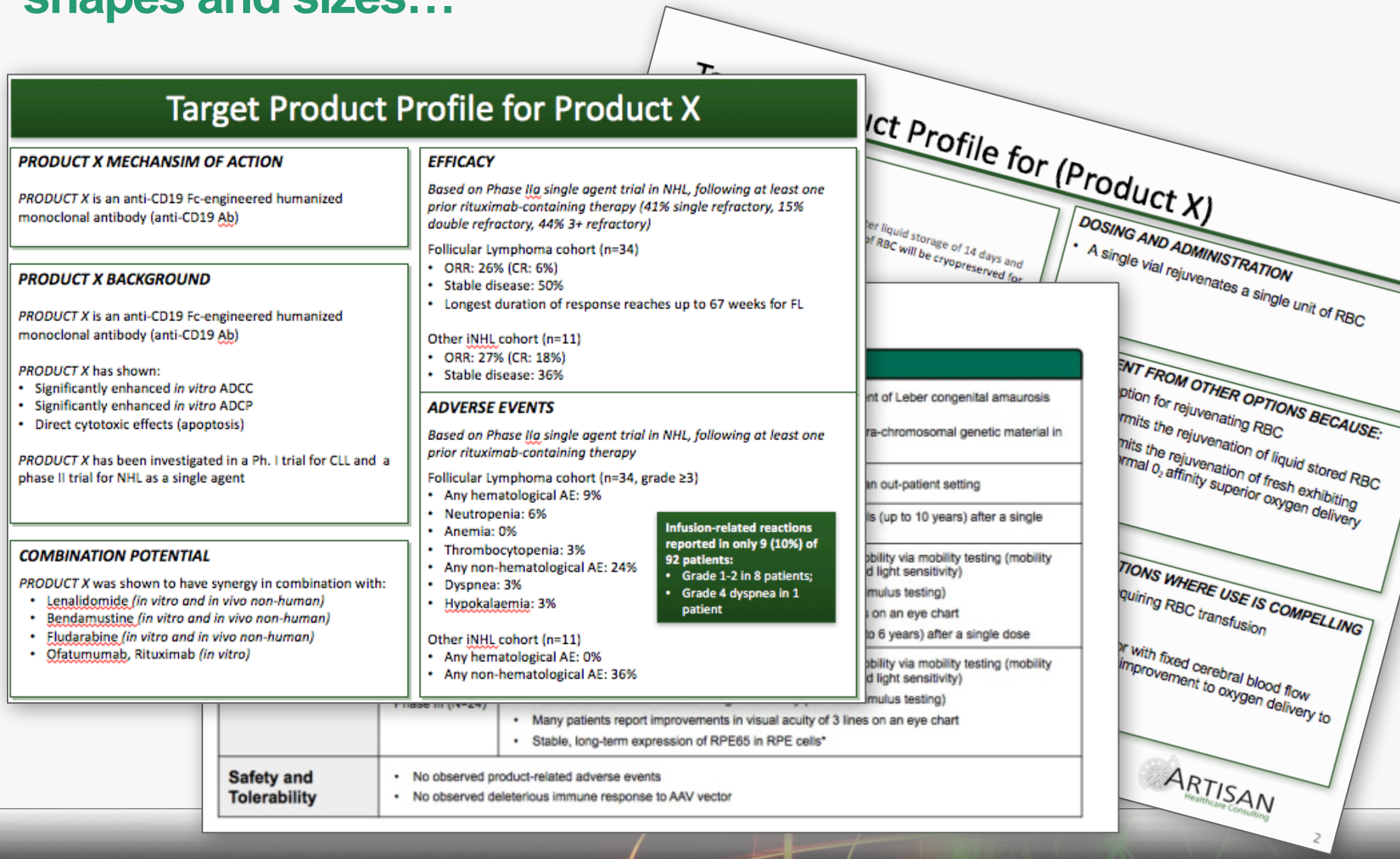
Target Product Profiles (TPP) can come in a variety of shapes and sizes…



Target Product Profile for Product X

PRODUCT X MECHANSIM OF ACTION

PRODUCT X is an anti-CD19 Fc-engineered humanized monoclonal antibody (anti-CD19 Ab)

PRODUCT X BACKGROUND

PRODUCT X is an anti-CD19 Fc-engineered humanized monoclonal antibody (anti-CD19 Ab)

PRODUCT X has shown:
- Significantly enhanced *in vitro* ADCC
- Significantly enhanced *in vitro* ADCP
- Direct cytotoxic effects (apoptosis)

PRODUCT X has been investigated in a Ph. I trial for CLL and a phase II trial for NHL as a single agent

COMBINATION POTENTIAL

PRODUCT X was shown to have synergy in combination with:
- Lenalidomide *(in vitro and in vivo non-human)*
- Bendamustine *(in vitro and in vivo non-human)*
- Fludarabine *(in vitro and in vivo non-human)*
- Ofatumumab, Rituximab *(in vitro)*

EFFICACY

Based on Phase IIa single agent trial in NHL, following at least one prior rituximab-containing therapy (41% single refractory, 15% double refractory, 44% 3+ refractory)

Follicular Lymphoma cohort (n=34)
- ORR: 26% (CR: 6%)
- Stable disease: 50%
- Longest duration of response reaches up to 67 weeks for FL

Other iNHL cohort (n=11)
- ORR: 27% (CR: 18%)
- Stable disease: 36%

ADVERSE EVENTS

Based on Phase IIa single agent trial in NHL, following at least one prior rituximab-containing therapy

Follicular Lymphoma cohort (n=34, grade ≥3)
- Any hematological AE: 9%
- Neutropenia: 6%
- Anemia: 0%
- Thrombocytopenia: 3%
- Any non-hematological AE: 24%
- Dyspnea: 3%
- Hypokalaemia: 3%

Infusion-related reactions reported in only 9 (10%) of 92 patients:
- **Grade 1-2 in 8 patients;**
- **Grade 4 dyspnea in 1 patient**

Other iNHL cohort (n=11)
- Any hematological AE: 0%
- Any non-hematological AE: 36%

…ct Profile for (Product X)

…er liquid storage of 14 days and …f RBC will be cryopreserved for

DOSING AND ADMINISTRATION
- A single vial rejuvenates a single unit of RBC

…NT FROM OTHER OPTIONS BECAUSE:
- …ption for rejuvenating RBC
- …rmits the rejuvenation of liquid stored RBC
- …mits the rejuvenation of fresh exhibiting …rmal O_2 affinity superior oxygen delivery

…TIONS WHERE USE IS COMPELLING
- …quiring RBC transfusion
- …r with fixed cerebral blood flow …improvement to oxygen delivery to

…nt of Leber congenital amaurosis

…ra-chromosomal genetic material in

…n out-patient setting

…s (up to 10 years) after a single

…bility via mobility testing (mobility …d light sensitivity)

…mulus testing)

… on an eye chart

…o 6 years) after a single dose

…bility via mobility testing (mobility …d light sensitivity)

…mulus testing)

- Many patients report improvements in visual acuity of 3 lines on an eye chart
- Stable, long-term expression of RPE65 in RPE cells*

Safety and Tolerability	• No observed product-related adverse events • No observed deleterious immune response to AAV vector

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2

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A TPP should be

- ☑ A **living** tool that outlines desired product characteristics and benefits
- ☑ **Collectively owned** by the Product Team
- ☑ **Specific** enough to provide a clear vision and focus to drive commercial and clinical success
- ☑ A **clear and concise summary** of what would be a real, successful product

A TPP shouldn't be

- ☒ A **static document** that is updated once during the year and not referred to again
- ☒ A document **owned by a singular function**
- ☒ A **lengthy document** detailing the rationale for each data point

Key components of a TPP

STRATEGIC INTENT	Product X demonstrates clinically meaningful and statistically significant superiority as a single agent in survival (PFS and OS), clinical benefit, and time to progression compared to Comparator X in [indication], and will benefit patients unable to receive [competitive set A].	**LAUNCH** 20XX(US/JP), 20XX(EU) **LoE** 20XX(US), 20XX(EU/JP)

TARGET INDICATION

Treatment of adults with advanced [disease] after failure of at least one [competitive set B] standard of care therapy. [Competitive Set B] may include any of Competitor A, B, C, or C, or others approved at time of P3 trial initiation).

KEY EFFICACY CLAIMS

Target Product X Endpoints and Efficacy:

- ☐ **CBR:** ***30% improvement*** vs. approved SOC at time of pivotal trial initiation; Target**:** 65-80% CBR (4%PR) vs. 50-68%
- ☐ **mPFS:** Minimum ***3 month improvement*** vs. comparator (Comparator X); Target**:** ≥ 8 mos. vs. 5 mos.
- ☐ **mOS:** Minimum ***30% improvement*** vs. comparator (Comparator X); Target**:** ≥ 20 mos. vs. 15 mos.

Ph1a Efficacy Data:**

CBR = 67%

KEY SAFETY CLAIMS

Target Product X Safety Profile:

- ☐ All grades pneumonitis 8%; 2% Gr3/4
- ☐ **All grades mucositis < mTOR; 4% Gr3/4**
- ☐ All grades aphthous ulcers ≤ mTOR; ≤ 5% Gr3 stomatitis.
- ☐ All grades ____ ≥ 50% incidence
- ☐ Potential Black Box Warning:

Ph1a Safety Data:**

Peripheral neuropathy (Gr3/4) <10%
Pneumonitis (Gr3/4) < 2%

DOSING AND ADMINISTRATION

Findings from Dose Escalation:

- ☐ No clinically significant effect on QTc interval
- ☐ Clinically manageable drug-drug interactions
- ☐ Daily or weekly administration
- ☐ Take on empty stomach 1 hour before/2 hours after meals
- ☐ Regimens: 30mg QW for [indication] Ph2

RP2D:

30mg QW

PAYER VALUE PROPOSITION

Product X significantly improves survival and demonstrates significantly ***less pneumonitis and complications of pneumonitis*** without worsening overall QoL or [indication] symptoms when ***compared with Competitive Set A***.

VALUE ATTRIBUTES

Patient Reported Outcomes

- ☐ HRQoL global, function, disease impact, and symptom-based
- ☐ Patient-reported ratings of health status

Economic/System Efficiency Impacts

- ☐ Budget Impact measures
- ☐ Health resource utilization (Hospital. rate)
- ☐ Treatment Pattern Implications (Sequencing /dose modifications with other branded tx)
- ☐ No CDx required

CLINICAL DIFFERENTIATION: Inhibition of [Mechanism] will provide a significant efficacy benefit and clearly improve pneumonitis safety/tolerability challenges vs current SoC.

COMMERCIAL VALUE DRIVERS: Reducing hospital costs while showing a 30% improvement in PFS will drive economic value; investment in earlier lines of therapy should begin ASAP to optimize compound value.

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DOSING AND ADMINISTRATION

Clearly state the **most compelling benefits** your compound will deliver to a **clearly defined population**.

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- ☐ Patient-reported ratings of health status

e/System Efficiency Impacts

measures

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- ☐ Treatment Pattern Implications (Sequencing /dose modifications with other branded tx)
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Articulate your desired indication to be included in the label.

Will trial protocol result in the indication you want?

CLINICAL DIFFERENTIATION: Inhibition of [Mechanism] will provide a significant efficacy benefit and clearly improve pneumonitis safety/tolerability challenges vs current SoC.

COMMERCIAL VALUE DRIVERS: Reducing hospital costs while showing a 30% improvement in PFS will drive economic value; investment in earlier lines of therapy should begin ASAP to optimize compound value.

Leverage competitive intelligence and available clinical data to select efficacy target, ideally with % improvement over current SOCs

KEY EFFICACY CLAIMS	KEY SAFETY CLAIMS	DOSING AND ADMINISTRATION
Target Product X Endpoints and Efficacy: ☐ **CBR:** ***30% improvement*** vs. approved SOC at time of pivotal trial initiation; Target**:** 65-80% CBR (4%PR) vs. 50-68% ☐ **mPFS:** Minimum ***3 month improvement*** vs. comparator (Comparator X); Target**:** ≥ 8 mos. vs. 5 mos. ☐ **mOS:** Minimum ***30% improvement*** vs. comparator (Comparator X); Target**:** ≥ 20 mos. vs. 15 mos.	**Target Product X Safety Profile:** ☐ All grades pneumonitis 8%; 2% Gr3/4 ☐ **All grades mucositis < mTOR; 4% Gr3/4** ☐ All grades aphthous ulcers ≤ mTOR; ≤ 5% Gr3 stomatitis. ☐ All grades ____ ≥ 50% incidence ☐ Potential Black Box Warning:	**Findings from Dose Escalation:** ☐ No clinically significant effect on QTc interval ☐ Clinically manageable drug-drug interactions ☐ Daily or weekly administration ☐ Take on empty stomach 1 hour before/2 hours after meals ☐ Regimens: 30mg QW for [indication] Ph2
Ph1a Efficacy Data:** CBR = 67%	**Ph1a Safety Data**:** Peripheral neuropathy (Gr3/4) <10% Pneumonitis (Gr3/4) < 2%	**RP2D:** 30mg QW

Repeat for Safety and Dosage/Administration

Key components of a TPP

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Pneumonitis (Gr3/4) < 2%

RP2D:
30mg QW

Unless you are in a cash pay market, the **payer value proposition** needs to be articulated early on, as well as the endpoints/benefits needed **demonstrate the value**.

PAYER VALUE PROPOSITION	VALUE ATTRIBUTES	
Product X significantly improves survival and demonstrates significantly ***less pneumonitis and complications of pneumonitis*** without worsening overall QoL or [indication] symptoms when ***compared with Competitive Set A***.	**Patient Reported Outcomes** ☐ HRQoL global, function, disease impact, and symptom-based ☐ Patient-reported ratings of health status	**Economic/System Efficiency Impacts** ☐ Budget Impact measures ☐ Health resource utilization (Hospital. rate) ☐ Treatment Pattern Implications (Sequencing /dose modifications with other branded tx) ☐ No CDx required

CLINICAL DIFFERENTIATION: Inhibition of [Mechanism] will provide a significant efficacy benefit and clearly improve pneumonitis safety/tolerability challenges vs current SoC.

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Ph1a CBR =	3/4) <10%	**RP2D:** 30mg QW

VALUE ATTRIBUTES

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Economic/System Efficiency Impacts
- ☐ Budget Impact measures
- ☐ Health resource utilization (Hospital. rate)
- ☐ Treatment Pattern Implications (Sequencing /dose modifications with other branded tx)
- ☐ No CDx required

Clinical differentiation
Clarity and specificity are important here

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DOSING AND ADMINISTRATION

after

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Patient Reported

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COMMERCIAL VALUE DRIVERS: Reducing hospital costs while showing a 30% improvement in PFS will drive economic value; investment in earlier lines of therapy should begin ASAP to optimize compound value.

Commercial value drivers

What are the key economic drivers for the compound? These should be identified and quantified early in development

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Set the Stage

Using the TPP to increase engagement and alignment with key stakeholders

- Because the TPP requires input and alignment from cross-functional members of the Product Team, ***disagreements will arise***
- **Remember**: The key to successful TPP development is cross-functional stakeholder involvement. TPPs developed outside this process will not have significant utility

Before you begin

- Secure senior management **explicit** buy-in and direction on how the TPP will be used outside of the Product Team and why
- Ensure **all members** of the Product Team know how the TPP will be used, both within the team and without

Best Practices

Using the TPP to increase engagement and alignment with key stakeholders

- You need to understand the big decisions or deliverables that your cross-functional counterparts are responsible for
- You need to determine which of those deliverables require in some shape or form information from the TPP
- You need to position yourself as a resource to help them have the best information possible. Get the best information you can and share it with the team!
- You need to come to GPT and other meetings armed with real information and insights for that compound in that disease, your gut or "experience" won't cut it
- Demonstrate that the TPP is optimizing the chances for a product's success

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1. Why is a TPP important?
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4. **Determine the right data needed to inform TPP discussions**
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Determine the right data needed to inform TPP discussions and what NPP teams need to be ready to discuss

TPP aspects that NPP should be prepared to share	Potential sources of information
Efficacy and safety benchmarking data	Clinicaltrials.gov, package inserts, PubMed, press releases, Kantar or other syndicated data
Target indication(s)	KOL discussions, trial investigators, internal discussions, Kantar, WHO, National Health Surveys
Clinically meaningful endpoints and corresponding thresholds	Current and pipeline clinical benchmarks, KOLs, payers, Market Access colleagues
Market access - value considerations and endpoints	Market Access colleagues, recent access decisions in target countries, HEOR model reviews
Compound differentiation	Patient journey, patient research insight, internal discussions, benchmarking data
Pricing	Medicare ASP files, press releases, commercial pricing subscription services

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Understand how a TPP should evolve over time

A TPP should **evolve** as compounds move into later stages of development

- ➔ Without clinical data, clinical, safety, and dosage/administration statements may be analog-based
- ➔ KOLs will provide input on the magnitude of benefit needed for clinical relevance
- ➔ Later in development, TPP values will be driven by real clinical data and other customer insights

KEY EFFICACY CLAIMS	KEY SAFETY CLAIMS	DOSING AND ADMINISTRATION
Target Product X Endpoints and Efficacy: ☐ **CBR: 30% improvement** vs. approved SOC at time of trial initiation ☐ **mPFS**: Minimum **3 month improvement** vs. approved SOC at time of trial initiation ☐ **mOS**: Minimum **30% improvement** vs. approved SOC at time of trial initiation	**Target Product X Safety Profile:** ☐ Overall incidence of pneumonitis ≤10%; ≤2% Gr3/4 ☐ Overall incidence of mucositis, oral stomatitis, aphthous ulcers ≤ mTOR and ≤ 5% incidence of Gr3 stomatitis.	**Target Product X Dosing** ☐ Daily or weekly administration ☐ IV: QW or QD
Benchmark Efficacy Data: ☐ **ORR/TTP**: 50-63% ☐ **mPFS**: 3-5 mos. ☐ **mOS**: 12-15 mos.	**AE Thresholds to Monitor[2]** ☐ Pneumonitis ≤15%; ≤5% Gr3/4 ☐ Hyperglycemia ≤15%; ≤5% Gr3/4 ☐ Stomatitis ≤60%; ≤5% Gr3/4	**Competitor Dosing** ☐ **Oral**: 1-2 tablets 1x/day ☐ **IV**: QW/Q2W/Q3W]

Understand how a TPP should evolve over time

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KEY EFFICACY CLAIMS	KEY SAFETY CLAIMS	DOSING AND ADMINISTRATION
Target Product X Endpoints and Efficacy: ☐ **CBR: 30% improvement** vs. approved SOC at time of trial initiation ☐ **mPFS**: Minimum **3 month improvement** vs. approved SOC at time of trial initiation ☐ **mOS**: Minimum **30% improvement** vs. approved SOC at time of trial initiation	**Target Product X Safety Profile:** ☐ Overall incidence of pneumonitis ≤10%; ≤2% Gr3/4 ☐ Overall incidence of mucositis, oral stomatitis, aphthous ulcers ≤ mTOR and ≤ 5% incidence of Gr3 stomatitis.	**Target Product X Dosing** ☐ Daily or weekly administration ☐ IV: QW or QD
Benchmark Efficacy Data: ☐ **ORR/TTP**: 50-63% ☐ **mPFS**: 3-5 mos. ☐ **mOS**: 12-15 mos.	**AE Thresholds to Monitor[2]** ☐ Pneumonitis ≤15%; ≤5% Gr3/4 ☐ Hyperglycemia ≤15%; ≤5% Gr3/4 ☐ Stomatitis ≤60%; ≤5% Gr3/4	**Competitor Dosing** ☐ **Oral**: 1-2 tablets 1x/day ☐ **IV**: QW/Q2W/Q3W]

PAYER VALUE CONSIDERATIONS

- ☐ Market will be a mix of generic (Comparator X) and branded competitors upon on the launch of Product X
- ☐ Product X is likely to have a limited budget impact, given the relatively small market in later lines of [Indication] therapy

- ↑ Market Access considerations will also evolve from considerations to endpoints

Understand how a TPP should evolve over time

STRATEGIC INTENT: Product X demonstrates clinically meaningful and statistically significant superiority as a single agent in survival (PFS and OS), clinical benefit, and time to progression compared to Comparator X in [indication], and will benefit patients unable to receive [competitive set A].

LAUNCH 20XX(US/JP), 20XX(EU)
LoE 20XX(US), 20XX(EU/JP)

TARGET INDICATION

Treatment of adults with advanced [disease] after failure of at least one [competitive set B] standard of care therapy. … approved at time of P3 trial initiation).

All components of the TPP should be reviewed as new competitors enter and clinical data emerge

KEY EFFICACY CLAIMS

Target Product X Endpoints and Efficacy:
- CBR … vs. approved SOC at time of pivotal trial initiation; Target: 6…80% CBR (4%PF…) vs. 50-60%
- mPFS: Minimum *3 month improvement* vs. comparator (Comparator X); Target: ≥ 8 mos. vs. 5 mos.
- mOS: Minimum … vs. comparator (Comparator X); Target: ≥ 20 mos. vs. 15 mos.

Ph1a Efficacy Data**:
CBR = 6…%

KEY SAFETY CLAIMS

Target Product X Safety Profile:
- All grades pneumonitis …%; 2% Gr3/4
- All grades … < mTOR; …
- All grades aphthous ulcers ≤ mTOR; ≤ 5% Gr3 stomatitis.
- All grades ____ > 50% incidence
- Potential Black Box Warning

Ph1a Safety Data**:
Peripheral neuropathy (Gr3/4) …
Pneumonitis (Gr3/4) < 2…

DOSING AND ADMINISTRATION

Findings from Dose Escalation:
- No clinically significant effect on QTc interval
- Clinically manageable drug-drug interactions
- Daily or weekly administration
- Take on empty stomach 1 hour before/2 hours after meals
- Regimens: 30mg QW for [indication] Ph2

RP2D:
30mg QW

PAYER VALUE PROPOSITION

Product X significantly improves survival and demonstrates significantly *less pneumonitis and complications of pneumonitis* without worsening overall QoL or [indication] symptoms when *compared with Competitive Set A*.

VALUE ATTRIBUTES

Patient Reported Outcomes
- HRQoL global, function, disease impact, and symptom-based
- Patient-reported ratings of health status

Economic/System Efficiency Impacts
- Budget Impact measures
- Health resource utilization (Hospital. rate)
- Treatment Pattern Implications (Sequencing /dose modifications with other branded tx)
- No CDx required

CLINICAL DIFFERENTIATION: Inhibition of [Mechanism] will provide a significant efficacy benefit and clearly improve pneumonitis safety/tolerability challenges vs current SoC.

COMMERCIAL VALUE DRIVERS: Reducing hospital costs while showing a 30% improvement in PFS will drive economic value; investment in earlier lines of therapy should begin ASAP to optimize compound value.

About the Author

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About Me - Brad Payne

Brad Payne is a Partner with Artisan Healthcare Consulting. He has lead and delivered >100 consulting engagements across multiple therapeutic areas, including oncology, rare diseases, cardiovascular, and medical devices.

Areas of expertise include:

- Early commercial strategy development for biopharma and medical devices
- Go-to-launch support
- Broad L&A support, from BD strategy to short-term due diligence assessments
- Global market access strategy development for pipeline and late-stage products

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Prior to joining Artisan, Brad was a consultant at Trinity Partners, a pharmaceutical and biotech consulting firm, where he provided insight on forecasting projects, licensing and acquisition opportunities, and provided recommendations for managed care contracting strategies.

Brad graduated with a degree in economics Cum Laude from Harvard University and received an MBA from the W.P. Carey School of Business at Arizona State University.