White Space – Identifying highly innovative product opportunities

Looking beyond the pipeline to generate growth

November 2015

Roland Berger team supports pharma companies to identify, understand and internalize highly attractive product opportunities

White Space Objectives

1. **Identify promising development assets in selected focus areas, not yet covered by internal activities**

2. **Prioritize development candidates based on the client's criteria and strategic needs**

3. **Create in-depth understanding for selected candidates and detail most promising opportunities**

4. **Develop business case to finalize development and launch successfully in high potential markets**

5. **Close the deal and make it happen**

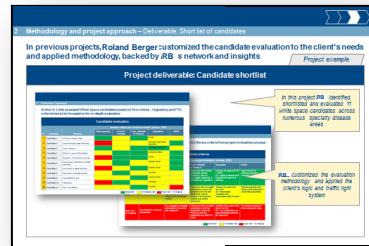*Identify, understand, internalize highly innovative opportunities*

Roland Berger White Space approach delivers tangible results that enable the client to achieve its aims

Database of late stage development candidates ✓
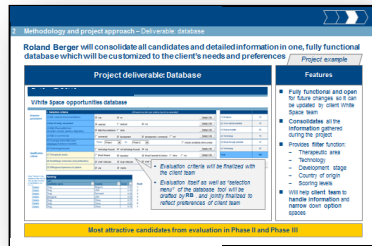
Short list of attractive candidates for BD ✓

In-depth assessment by expert capacities ✓
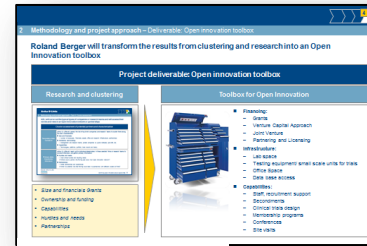
Business case and deal making support ✓

> Fully functional and open for future changes so it can be updated by client White Space team
> Consolidates all the information gathered during the project
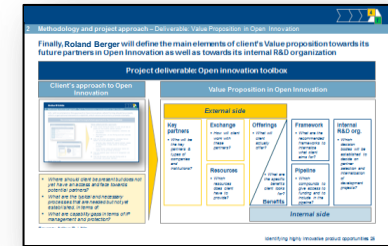> Will help client team to handle information and narrow down option spaces

> Shortlist of most attractive candidates for the client
  – Out of the pre-defined therapeutic scope and selected diseases/ areas
  – Evaluate along your criteria and individual strategic needs
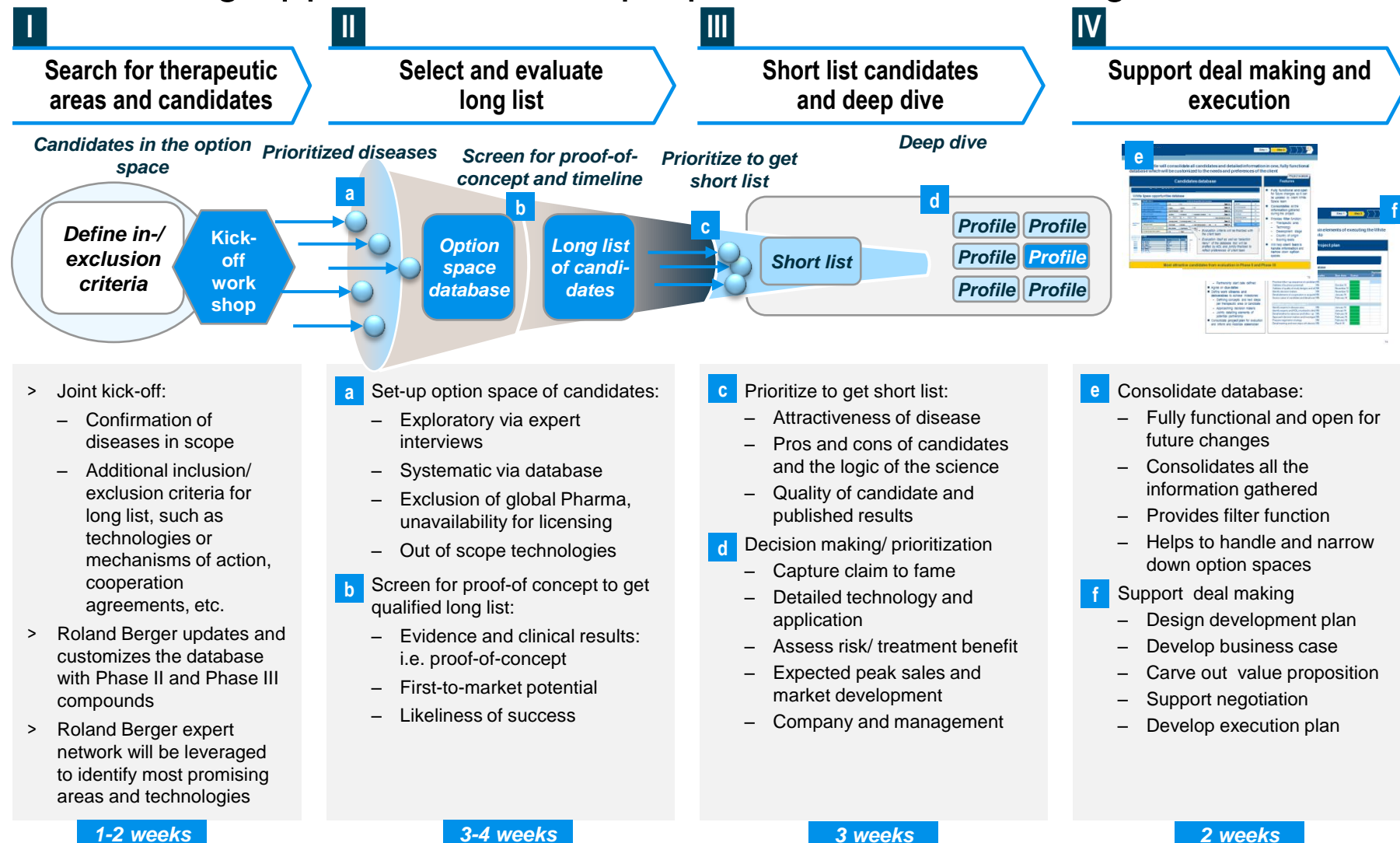 – Selection steps and applied criteria transparent

> Roland Berger will assess the short listed candidates by scientific and commercial criteria:
  > Feasibility of disease pathway
  > Robustness of clinical evidence
  > Regulatory pathway
  > Market access prospects
  > Commercial potential

> Roland Berger carves out the value proposition for the internalization, development and launch of the product
> Business case including robust assumptions and scenarios on price and volumes in target markets
> Negotiation and deal making support

In four phases, Roland Berger Team develops a qualified short list of in-licensing opportunities and prepares for deal making

I Search for therapeutic areas and candidates

II Select and evaluate long list

III Short list candidates and deep dive

IV Support deal making and execution

Candidates in the option space
Prioritized diseases
Screen for proof-of-concept and timeline
Prioritize to get short list
Deep dive

Define in-/exclusion criteria → **Kick-off work shop**

a

b Option space database | Long list of candi-dates

c Short list

d Profile Profile / Profile Profile / Profile Profile

e **f**

> Joint kick-off:
> – Confirmation of diseases in scope
> – Additional inclusion/ exclusion criteria for long list, such as technologies or mechanisms of action, cooperation agreements, etc.
> Roland Berger updates and customizes the database with Phase II and Phase III compounds
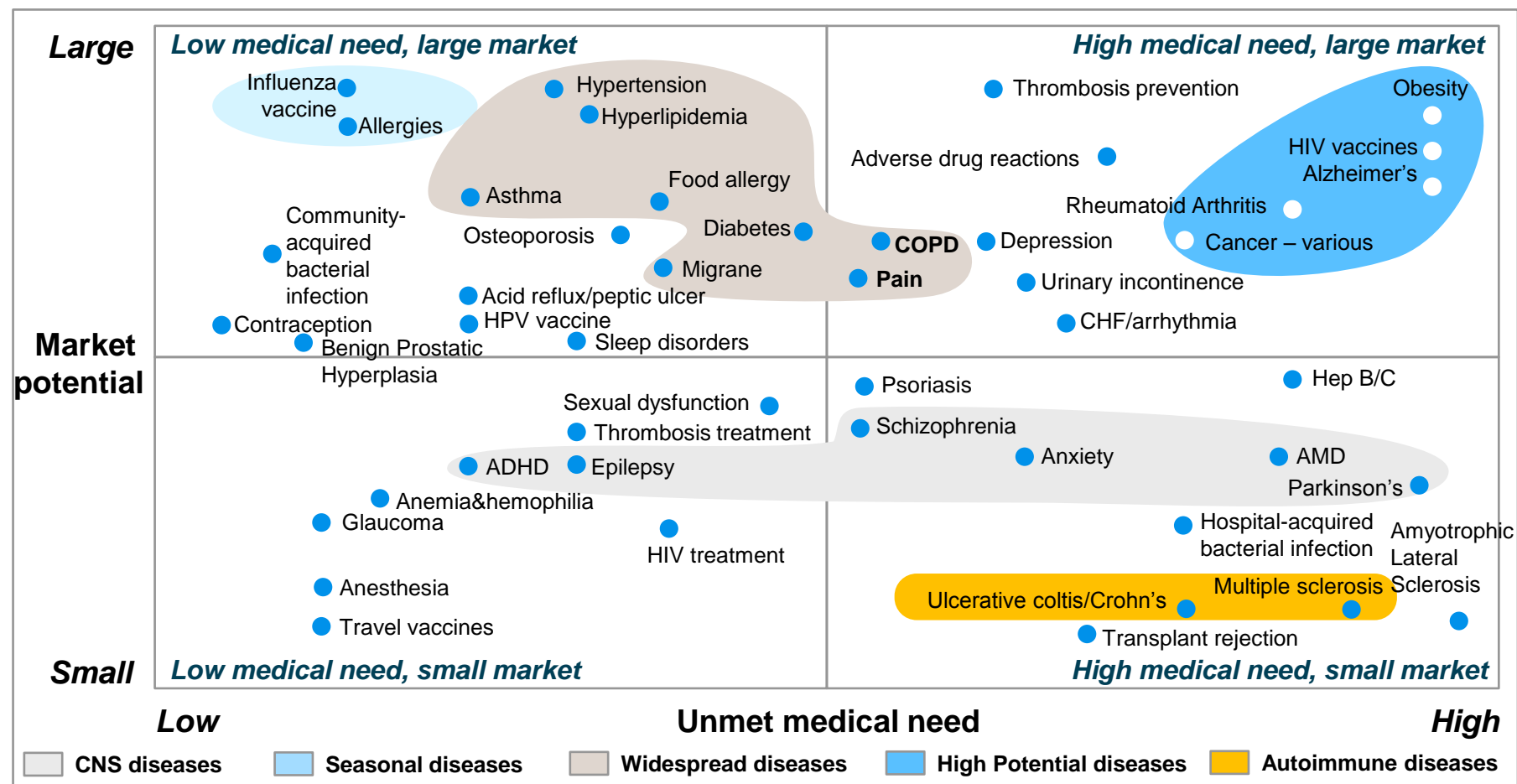> Roland Berger expert network will be leveraged to identify most promising areas and technologies

a Set-up option space of candidates:
– Exploratory via expert interviews
– Systematic via database
– Exclusion of global Pharma, unavailability for licensing
– Out of scope technologies

b Screen for proof-of concept to get qualified long list:
– Evidence and clinical results: i.e. proof-of-concept
– First-to-market potential
– Likeliness of success

c Prioritize to get short list:
– Attractiveness of disease
– Pros and cons of candidates and the logic of the science
– Quality of candidate and published results

d Decision making/ prioritization
– Capture claim to fame
– Detailed technology and application
– Assess risk/ treatment benefit
– Expected peak sales and market development
– Company and management

e Consolidate database:
– Fully functional and open for future changes
– Consolidates all the information gathered
– Provides filter function
– Helps to handle and narrow down option spaces

f Support deal making
– Design development plan
– Develop business case
– Carve out value proposition
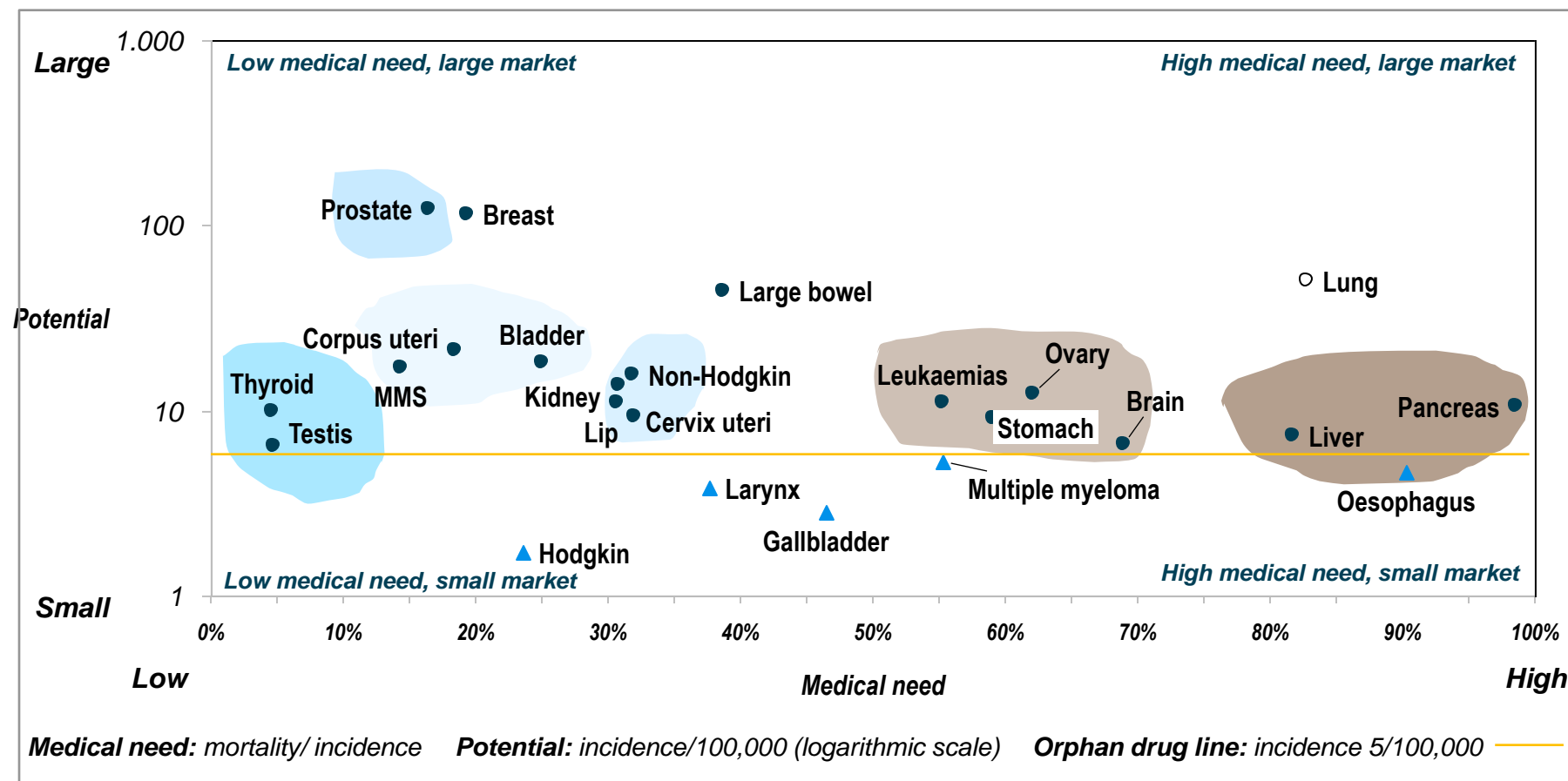– Support negotiation
– Develop execution plan

1-2 weeks | *3-4 weeks* | *3 weeks* | *2 weeks*

Option space can be clustered along indications on unmet medical need and market potential

Task 1: Disease option space

	Low	Unmet medical need	High

Large

Low medical need, large market
- Influenza vaccine
- Allergies
- Hypertension
- Hyperlipidemia
- Food allergy
- Asthma
- Osteoporosis
- Diabetes
- Community-acquired bacterial infection
- Migrane
- COPD
- Pain
- Acid reflux/peptic ulcer
- HPV vaccine
- Contraception
- Benign Prostatic Hyperplasia
- Sleep disorders

High medical need, large market
- Thrombosis prevention
- Obesity
- Adverse drug reactions
- HIV vaccines
- Alzheimer's
- Rheumatoid Arthritis
- Depression
- Cancer – various
- Urinary incontinence
- CHF/arrhythmia

Market potential

Low medical need, small market
- Sexual dysfunction
- Thrombosis treatment
- ADHD
- Epilepsy
- Anemia&hemophilia
- Glaucoma
- HIV treatment
- Anesthesia
- Travel vaccines

High medical need, small market
- Psoriasis
- Hep B/C
- Schizophrenia
- Anxiety
- AMD
- Parkinson's
- Hospital-acquired bacterial infection
- Amyotrophic Lateral Sclerosis
- Ulcerative coltis/Crohn's
- Multiple sclerosis
- Transplant rejection

Small

CNS diseases	Seasonal diseases	Widespread diseases	High Potential diseases	Autoimmune diseases

Cancer types can be clustered along incidence and mortality

Example: Oncology option space

Cancer types in detail: Lung incl. trachea & bronchus; MMS: Malignant melanoma of skin; Kidney incl. renal pelvis & ureter; Lip oral cavity & pharynx; Liver & intrahepatic bile ducts; Gallbladder & biliary tract;

Both, Phase III and Phase II Pipeline stages contain thousands of development candidates

Disease option space – example clinical candidates

Option1: Phase III for all therapeutic areas

Therapeutic areas	Number of studies per disease	No. of diseases
Alimentary/metabolic	123	92
Blood and clotting	43	32
Cancer	515	81
Cardiovascular	76	37
Dermatological	58	42
Genitourinary	47	38
Hormonal	12	11
Immunological	15	9
Infectious Disease	177	92
Miscellaneous	18	6
Musculoskeletal	72	50
Neurological	166	84
Parasitic	6	5
Respiratory	60	26
Sensory	70	37
Grand Total	1,458	642

> One candidate may have more than one ongoing study

> On average, one candidate is tested in 2 studies

Option2: Phase II by therapeutic areas

Therapeutic areas	Number of studies per disease	No. of diseases
Alimentary/metabolic	302	92
Blood and clotting	75	32
Cancer	1,222	81
Cardiovascular	170	37
Dermatological	154	42
Genitourinary	127	38
Hormonal	16	11
Immunological	28	9
Infectious Disease	326	92
Miscellaneous	36	6
Musculoskeletal	171	50
Neurological	421	84
Parasitic	14	5
Respiratory	183	26
Sensory	108	37
Grand Total	3,353	642

> No. of diseases represents the total number of diseases that is summarized in each category

Client and Roland Berger jointly identify focus disease areas

Roland Berger will assess the identified opportunities against rigid criteria of clinical evidence

Screen for proof-of-concept and timeline

Prioritized diseases

a

Screen for proof-of-concept and timeline

b

Option space database

Long list of candi-dates

...tize to get ...rt list

Short list

d

Profile Profile
Profile Profile
Profile Profile

b
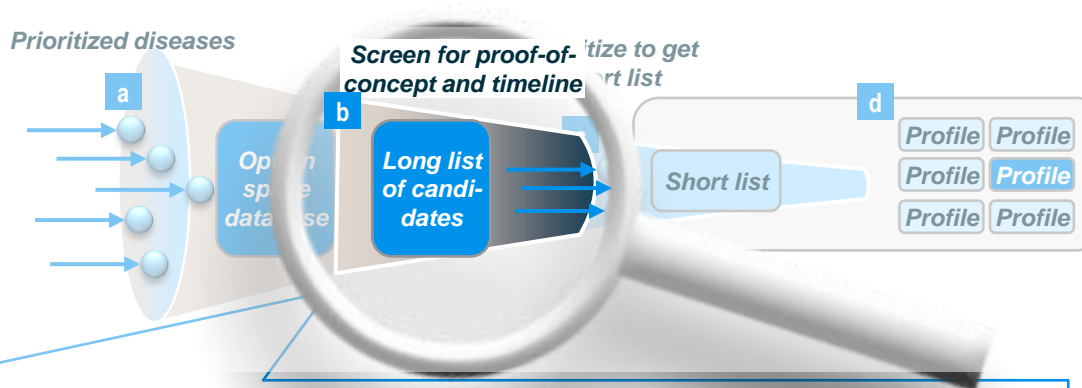
Screen for proof-of concept to get long list:

> Clinical studies must achieve a statistically significant difference in the primary endpoint of a validated surrogate parameter in a randomized, double-blind, controlled study

> In addition the clinical studies should meet the following proposed criteria:

‒ Was a comparison with a positive comparator included?

‒ Was more than one dose tested?

‒ Has Phase III trial been initiated?

‒ Is there first-to-market potential?

‒ How valid is the likeliness of success?

‒ Is the candidates development pathway within client's timeline?

Long list of candidates

Each candidate that meets the proof-of-concept criteria will be profiled and enters the long list

Validation of clinical studies for diseases set in scope

Workflow

> Identify all relevant candidates as well as phase II and phase III clinical studies that are set in scope for a specific disease

> Research the disease and actual treatment pathway of candidate

> Validate that there actually is a study for the disease phase II or phase III

> Validate if the license is not yet taken by a Global Player or another company and availability for focus countries

> Validate if there was a proof-of-concept (PoC) according to requirement provided by client team

Long List: Candidate Profiles

From Roland Berger's experience, approximately 10% of candidates will meet strict PoC criteria

In a further step, Roland Berger team prioritizes candidates on attractiveness of disease, strengths, weaknesses and science logic

Screen for proof-of-concept and timeline
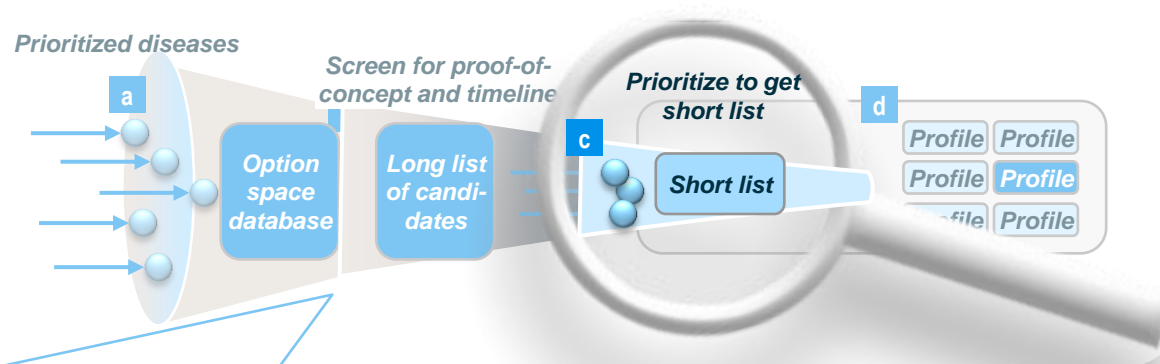
Prioritized diseases

Screen for proof-of-concept and timeline

Prioritize to get short list

a

c

d

Option space database

Long list of candi-dates

Short list

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c

Prioritize to get short list

> **Attractiveness of disease**

> **Pros and cons of candidates and the logic of the science**

> **Quality of candidate and published results**

This information will be researched by Roland Berger and extensively added to by the external expert network

Finally, evaluation is based on five areas: Candidate, readiness, disease, market attractiveness, company and team

Candidate Evaluation
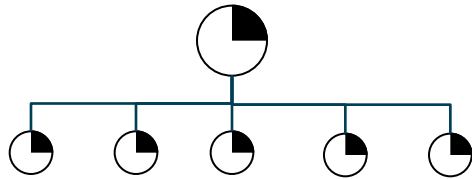
Summary of evaluation

> Summarizes the assessment of the five areas in scope

> Lists most important aspects

> Add-ups the assessment per area into an overall evaluation

Candidate evaluation

> Summary on candidate
> Expert evaluation
> Pros and cons
> Market Access
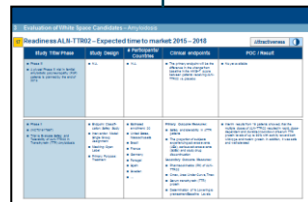
Readiness

> Summary of most important trials
> Endpoints/ POC
> Resulting time to market and R&D expenditure
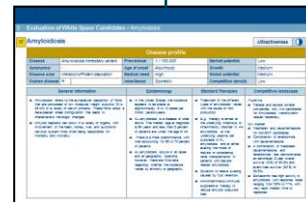
Disease evaluation

> Summary of disease characterization
> Prevalence
> Standard treatment and costs
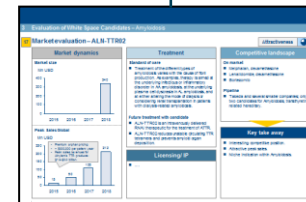
Market attractiveness

> Market size estimate
> Estimated peak sales
> Competition
> Licensing status

Company and team

> Company profile
> Key people
> Pipeline & partnerships
> Latest news

Roland Berger will consolidate all candidates and detail information in one, fully functional database

Database

Project deliverable: Database

Features

> Fully functional and open for future changes so it can be updated by client White Space team

> Consolidates all the information gathered during the project

> Provides filter function:
  – Therapeutic area
  – Technology
  – Development stage
  – Country of origin
  – Scoring levels

> Will help client team to handle information and narrow down option spaces

This tool and the evaluated compound and related companies also build the foundation for Phase 4 in the project

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