

# Access and Affordability Program Implementation – Update



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# Overview

## Context

- Prop 14, SAF Goal 5, and Aug 2023 AAWG feedback

## Our Process

- Tool development
  - A&A Checklist
  - Evaluation Rubric
  - Draft Guidance Document
- Tool Integration into Preclinical and Clinical programs
- AAWG Input

## Updates since 04/30/25 AAWG meeting

# Background – Why A&A Planning Must Be Embedded Early

## Strategic Imperative from Proposition 14

- Ensure that therapies developed with public funds **are accessible and affordable for all Californians**, particularly underserved populations

## Feedback from AAWG (August 2024)

- Consensus: A&A operationalize @ both programmatic and project levels
- Broad agreement that access strategies must align with the *stage of development*
- Need for **consistent expectations** and clearer guidelines across CIRM funding stages

## AAWG Toolkit Review and Approval (April 2025)

- Approved A&A Toolkit and Guidance Documents for implementation of A&A in CIRM Funding Programs

# CIRM's Impact Goals

## Accelerating Discovery & Translation

1. **Catalyze** the identification and validation of at least 4 novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California
2. **Accelerate** development and utilization of 5-8 technologies that have the potential to improve safety, efficacy, and/or quality of cell and gene therapies

## Cell & Gene Therapy Approvals

3. **Advance** 4-7 rare disease projects to BLA
4. **Propel** 15-20 therapies targeting diseases affecting Californians to late-stage trials

## Accessibility & Affordability of CIRM-Funded Cell & Gene Therapies

5. **Ensure** that every BLA-ready program has a strategy for access and affordability

## Diverse Workforce Development

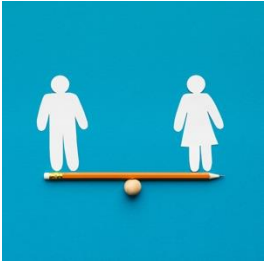
6. **Bolster** CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine

# Framing the Challenge – Key Questions and Considerations

## Guiding Questions Raised by the AAWG

- What *stage-appropriate* A&A activities should be required, and when?
- How can CIRM leverage its infrastructure to *incentivize planning early* while ensuring feasibility and proportionality?
- What mechanisms and metrics are needed to *track and support* execution of A&A strategies?

# Access and Affordability Definitions



## Accessibility

The ability of individuals to obtain and use healthcare services, information, and resources



## Affordability

An individual's ability to afford treatment, testing and other health care costs



## Commercialization

The process of bringing a product to market, encompassing everything from R&D to manufacturing, marketing, and distribution



## Market Access

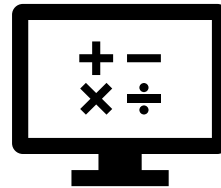
Ensuring the right patients receive the right treatments at the right time by effectively navigating healthcare payers, reimbursement policies, and pricing strategies

# A&A tools and resources being developed with BlueRidge



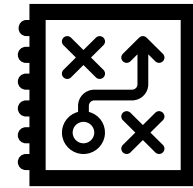
## Checklist

- Interactive list of phase-appropriate A&A activities as subset of commercialization activities
- Allows PDEV and CDEV funding mechanisms to create A&A review criteria



## Evaluation Rubric

- Excel tool to calculate a composite score for an applicant's A&A activities
- Embedded in review processes for CDEV funding mechanisms



## Guidance Documents

- Allows applicants, awardees, and reviewers to align on expectations

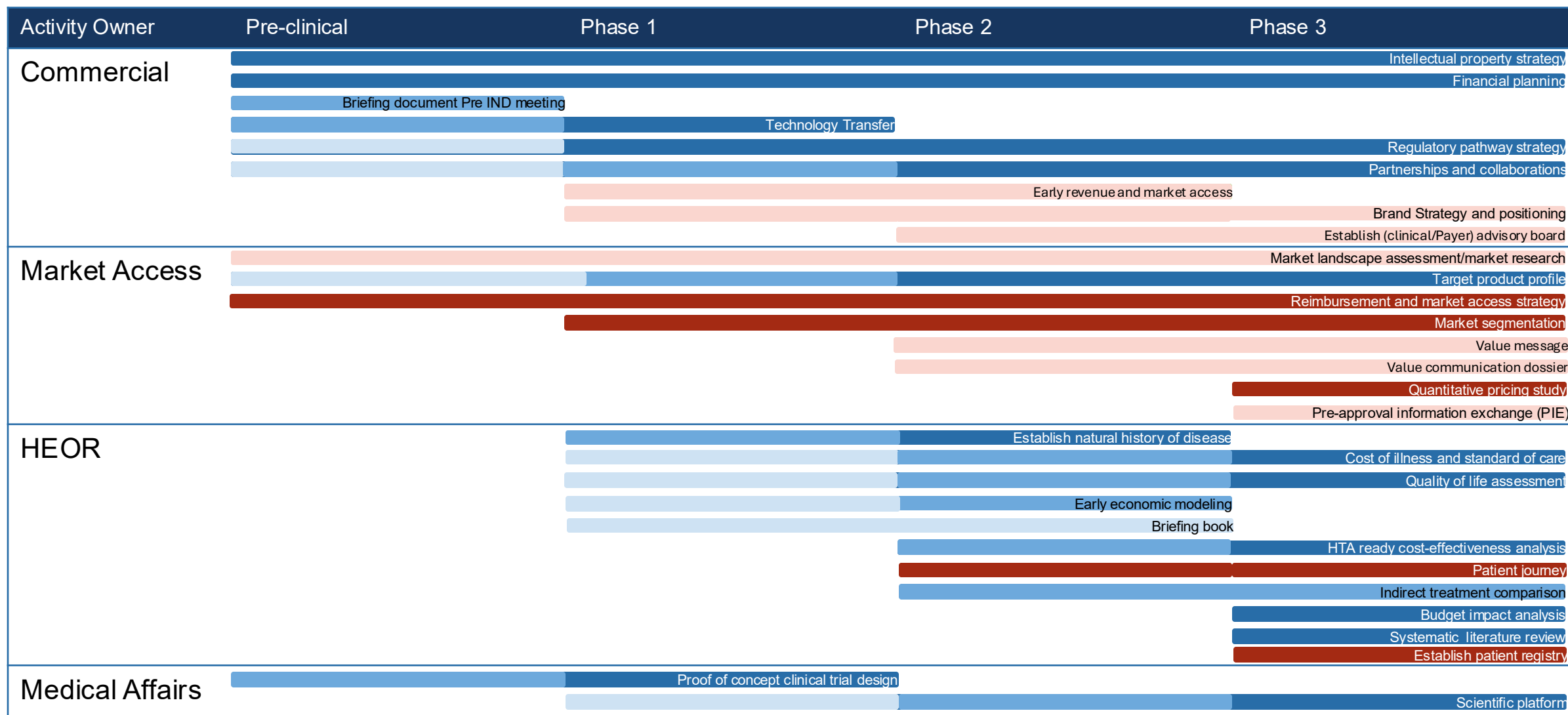
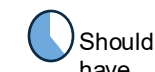
# **A&A Toolkit**

## **Preclinical and Clinical Programs**



# A&A in the Context of Commercialization Planning

Color Key



Designated as supporting access/affordability by BlueRidge

Designated as possibly supporting access/affordability

# The Rest of the Presentation Will Focus on A&A Activities

Activity Owner	Pre-clinical	Phase 1	Phase 2	Phase 3
Commercial				Intellectual property strategy
				Financial planning
	Briefing document Pre IND meeting			
	Technology Transfer			
				Regulatory pathway strategy
				Partnerships and collaborations
				Early revenue and market access
				Brand Strategy and positioning
Market Access				Establish (clinical/Payer) advisory board
				Market landscape assessment/market research
				Target product profile
				Reimbursement and market access strategy
				Market segmentation
				Value message
				Value communication dossier
				Quantitative pricing study
HEOR				Pre-approval information exchange (PIE)
				Establish natural history of disease
				Cost of illness and standard of care
				Quality of life assessment
				Early economic modeling
				Briefing book
				HTA ready cost-effectiveness analysis
				Patient journey
Medical Affairs				Indirect treatment comparison
				Budget impact analysis
				Systematic literature review
				Establish patient registry
	Proof of concept clinical trial design			Scientific platform

Designated as supporting access/affordability by BlueRidge

Designated as possibly supporting access/affordability

# Checklist: Stage-Appropriate Access and Affordability Activities

	PDEV		CLIN2: Phase 1		CLIN2: Phase 2		CLIN2: Phase 3	
Activity name	Must Have at Entry	Award Milestone	Must Have at Entry	Award Milestone	Must Have at Entry	Award Milestone	Must Have at Entry	Award Milestone
Market landscape assessment/market research		✓	✓		✓		✓	
<b>Reimbursement and market access strategy*</b>		✓		✓	✓		✓	
Early revenue and market forecast				✓	✓		✓	
Brand strategy and positioning						✓	✓	
<b>Market segmentation*</b>						✓	✓	
Establish (clinical and payer) advisory board						✓	✓	
Value message						✓	✓	
<b>Quantitative pricing studies*</b>						✓	✓	
Value communication dossiers						✓	✓	
<b>Establish patient registry/Risk-evaluation strategy (post launch)*</b>						✓	✓	
<b>Patient journey*</b>								✓
Pre-approval information exchange (PIE)								✓

\*Activities with strong impact supporting A&A

# A&A Implementation Overview – Preclinical

	PDEV
<b>Application Requirements</b>	<ul style="list-style-type: none"><li>• Describe A&amp;A activity progress prior to date</li><li>• Describe plan for achievement of stage-appropriate A&amp;A activities over course of PDEV award</li></ul>
<b>Application Components</b>	A&A checklist items incorporated into existing application proposal sections
<b>Reviewer</b>	Grants Working Group (GWG) scientific review
<b>Scoring &amp; Evaluation</b>	Assessment of A&A activities incorporated into GWG review criteria (significance and project planning) and overall GWG score
<b>Review Outcome</b>	<ul style="list-style-type: none"><li>• Informs GWG recommendation, CIRM team recommendation and ARS approval</li><li>• GWG feedback provided to applicant</li><li>• Informs CIRM management of approved awards</li></ul>

# A&A Implementation Overview – Clinical

	CLIN2
<b>Application Requirement</b>	<ul style="list-style-type: none"><li>• Describe completion of A&amp;A checklist activities</li><li>• Plan for achievement of stage-appropriate checklist activities</li></ul>
<b>Application Components</b>	Organized by A&A checklist for completed and planned activities for each trial phase
<b>Initial CIRM review</b>	Assess completeness of stage-specific checklist items in application
<b>GWG Review</b>	A&A checklist review conducted by expert specialist consultant
<b>Scoring &amp; Evaluation</b>	<ul style="list-style-type: none"><li>• Each activity scored for completeness and adequacy to yield a composite weighted score</li><li>• Qualitative assessments of A&amp;A activities and overall planning</li></ul>
<b>Outcome</b>	<ul style="list-style-type: none"><li>• Informs GWG recommendation, CIRM team recommendation and ARS approval</li><li>• Consultant feedback provided to applicant</li><li>• Informs CIRM management of approved awards</li></ul>

# Rubric

Composite score is a cumulative result of the overall status of A&A activities within 4 categories

## A&A\* activity score weighting

- A composite A&A activity score is calculated using the following formula:

- Composite A&A activity score:

$$\sum_{n=1}^n \left( \frac{(Priority\ Score)_{Activity\ n} \times (Raw\ Score)_{Activity\ n}}{5} \right)$$

- Note: the denominator is 5 so the top raw score assignment (raw score range 0-5) will be fully weighted



CIRM may use the composite scores to make funding decisions

An Excel tool was developed to facilitate calculating the composite score for A&A activities

Cell and Gene Therapy (CGT) Checklist for Commercialization Activities and Scoring Tool										Priority Level Key				Raw Score				Weighted Score			
										Low (Green)				Medium (Yellow)				High (Red)			
										0 (Not started)				1 (Reasonably planned)				2 (In process/partially completed)			
										3 (Completed but insufficient)				4 (Completed but needs updating)				5 (Completed in last 12 months)			
Development Stage	Phase 3																				
Activity number	Timing activity	Activity name	Activity type	Activity description	Benefits of undertaking activity	Barriers to executing activity	Overcoming barriers	Supports Access/Adopt	Pre-clinical	Phase 1	Phase 2	Phase 3	Raw Score	Raw Score Weight	Level of Priority	Weighted Score					
4	Pre-clinical/1/2/3	Market landscape assessment/market research	Market Access	Conducting detailed market research to assess the potential demand, competition, pricing strategies, and market gaps.	Understanding the size of the market, potential customers, unmet needs, and potential barriers to entry.	The cost of preclinical market research can be perceived as prohibitively expensive and very time consuming for small companies or startups and often there is only one person in the company with Accessing reliable evidence including real-world data to inform pricing and reimbursement decisions can be challenging.	Perform in-house research reaching out to providers/payers/advisory groups, and other key stakeholders.	Possible					2	0.5	3	1.5					
10	Pre-clinical/1/2/3	Reimbursement and market access strategy	Market Access	Planning for the product's reimbursement strategy by assessing the healthcare landscape, payer preferences, and pricing models.	Early understanding of the product's economic value to health systems and key stakeholders is important for successful adoption in the market.	These analyses are refined throughout the development process and useful for negotiating potential licensing agreements.	Utilize free online industry reports to inform Ensure market research is executed highlighting where the product fits into the clinical paradigm.	Yes					2	0.5	3	1.5					
12	Phase 1/2/3	Early revenue and market forecast	Commercialization	Develop early market and revenue forecasts using published evidence and qualitative primary research to understand the target population, peak market share, pricing and resulting revenues.	These analyses are refined throughout the development process and useful for negotiating potential licensing agreements.	Early forecasts will be rudimentary, and largely based on crude hypothetical assumptions.	Document key assumptions for early forecasts that are developed in-house - these assumptions can be validated or refined during subsequent market research.	Possible					2	0.5	3	1.5					
13	Phase 1/2/3	Brand strategy and positioning	Commercialization	Starting the process of brand development, including naming and positioning the drug in the marketplace.	lay the foundation for future value message development and what drivers of value are important to physicians, patients, payers and other stakeholders.	Early product positioning will be largely hypothesis driven given the lack of available/relevant evidence.	Ensure market research is executed highlighting where the product fits into the clinical paradigm; research with payers and providers will give insight into how the product will be perceived.	Possible					2	0.5	3	1.5					
14	Phase 1/2/3	Market segmentation	Market Access	Understand how a product is differentiated (e.g. unique potential customer segments/providers/payers) into groups based on different characteristics (e.g. geography, demographics, behaviors, etc.)	Key benefits include the ability to optimize marketing effort, enhance adoption and brand competitiveness.	Not understanding the product's place in Lack of data to be able to look at geography, demographics, and behavior of the different stakeholders.	Identify datasets that allow for identification of site of service and HCP prescribing behaviors.	Yes					2	0.5	3	1.5					
20	Phase 2/3	Establish (clinical and payer) advisory board	Commercialization	Identify key opinion leaders who could serve as advisors. Periodically seek feedback on clinical and economic evidence generation efforts.	Clinical and payer KOLs offer expert advice as the development program progresses through the clinical stages of development through commercial launch.	Identifying KOLs and logistics of identifying availability of multiple advisors with busy schedules.	Develop logistics to follow relevant clinical and local rules and laws when organizing an advisory board meeting.	Possible					2	0.5	3	1.5					
21	Phase 2/3	Value message	Market Access	Develop value messages that communicate key drivers of value, which is supported by evidence that help differentiate a therapeutic in the marketplace.	Value messages help clearly communicate the benefits of a drug to various stakeholders, including regulators, payers, healthcare providers, and patients. This ensures that everyone understands the benefits of a drug to various stakeholders.	Not incorporating key stakeholder input early in the evidence generation process (i.e. Phase 1) so that Phase 2 and pivotal trials contain the right data to support a strong value proposition.	Conduct early market research (i.e. pre-clinical or Phase 1) into the market landscape, unmet needs, and disease burden.	Possible					2	0.5	3	1.5					
23	Phase 2/3	Patient journey	HEOR	Develop a detailed pathway documenting the patient experience and healthcare service delivery from the point of a patient symptom on-set through diagnosis, treatment and management.	Developing a patient journey helps identifying pain points (unmet needs) and areas for improvement that a new treatment might alleviate and potentially lead to a more seamless and positive patient/provider experience.	Integrating and accessing data from various sources, such as electronic health records, patient surveys, and clinical trials, can be difficult.	Engage with experts with experience in integrating data from diverse sources to streamline the process.	Yes					2	0.5	2	1					
25	Phase 2/3	Value communication dossier	Market Access	Development of AMCP dossier for the US (pre-submission/initial approved reviews) and HTA submission dossiers for Ex-US markets.	As part of the technology assessment US payers typically request an AMCP dossier for new interventions.	Sensitivity to sharing pre-approval information (e.g. clinical study report, final label and launch pricing).	Ensure available evidence is collected (i.e. market research, SLK, TIC, model reports, clinical study report, pre-launch label, value messages).	Possible					2	0.5	3	1.5					
27	Phase 3	Quantitative pricing studies	Market Access	Conduct a robust conjoint analysis to understand the impact of product attributes have on price from the perspective of payers and other key stakeholders.	Understand the market acceptable price given demonstrated product attributes.	Quantitative pricing studies require a large sample size of participants and can be very costly to execute due to development and programming of a pricing and attribute survey. Also in late diseases it may not be aligned on the variables and outcomes to collect.	In real-world/disease disease areas leverage other research studies (e.g. CEA, BMA, advisory boards, qualitative pricing research) to inform pricing decisions.	Yes					2								
29	Phase 3/Registration	Establish patient registry/Risk-evaluation strategy (post-launch)	HEOR	Develop a registry for patients to collect long-term, real-world data on the safety, efficacy, and outcomes of CGT or best supportive care or usual care.	Enables monitoring of patients treated (or not treated) with a cell or gene therapy over the long term. Enables quantification and validation of long-term treatment effects including duration of effect on other.	Costly to design, implement and maintain, which for CGT therapies could be a barrier.	Develop a study plan, protocol and project plan that outlines the scope, purpose/objectives, timeline, patient populations, interventions, outcomes.	Yes					2								
30	Phase 2/3	Pre-approval information exchange (PIE)	Market Access	Develop a presentation to proactively share and educate payers in the US about certain health care economic and scientific information about a new emerging products.	Market readiness tool that is used to facilitate early market access conversations with payers before Food and Drug Administration (FDA) approval.	Requires internal process for regulatory review and approval of materials to be shared externally with payers and other HCEI audiences.	Ensure there is redundancy support for the HCEI to comply FDA regulations and socialization of best practices on sharing	Possible					2								
Activity Count										2	5	9	12								
Total CIRM Commercialization Score for Application																					
										</											

# Award Management

	PDEV	CLIN2
<b>Fundable Activities</b>	Both programs will fund stage-appropriate allowable A&A activities	
<b>Progress Reporting on all stage appropriate activities</b>	Awardee will report progress on A&A activities over course of award	
<b>Implementing milestones on A&amp;A activities</b>	<ul style="list-style-type: none"><li>• A&amp;A activities will be incorporated in award milestones</li><li>• Inadequate progress on milestones could impact continuation of award</li></ul>	
<b>Final Submission</b>	Award will submit final strategic planning report as part of final operational milestone	

## ***AAWG (04/30/2025) - Key points***

- **Broad support for early and consistent integration** of A&A planning
- **Scope** of activities **aligns with field standards** and **expectations** for comprehensive planning
- Execution will **require diverse expertise** across awardee teams
- **Recommend** CIRM provide **clear guidance** to applicants, reviewers, and the ICOC
- CIRM should **evaluate the completeness** of A&A plans during review and oversight



# Feedback implementation

## *Updates since AAWG meeting 4/30/25*

- PDEV and CDEV A&A **toolkit implemented**:
  - CLIN2 & PDEV **PAs** posted with **A&A requirements** section
  - A&A stage-appropriate activities **checklist** provided
  - **Guidance Document** released with A&A activities defined and strategies to accomplish each listed
- **Wide range of consultants engaged**