# [48] Quality compliance while conducting NIS studies: critical success factors assessment and the use of key performance indicators [1044]

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## BACKGROUND

Multi-country non-interventional studies (NIS) are vital for post-marketing surveillance, offering real-world evidence on medicine use, effectiveness, and safety for patient benefits. Following high-quality standards, as advised by regulatory authorities, guarantees dependable and traceable results. Existing regulations and guidelines stress transparency, scientific thinking, data quality, and statistical methods. Nevertheless, incorporating key performance indicators (KPIs) and critical success factors is essential to evaluate overall quality compliance in NIS.

## OBJECTIVES

This study aimed to explore the current gaps in multi-country NIS quality management and develop guidelines on the critical success factors and KPIs to harmonise the measurement of NIS quality compliance.

## METHODS

A comprehensive assessment of critical success factors in NIS was conducted by reviewing the quality requirements and KPIs outlined in the regulatory guidelines of the European Medicines Agency (EMA) and the Food and Drug Administration (FDA) agencies (Table 1). An evaluation of challenges associated with NIS preparation and execution was performed, two focus group discussions were conducted involving relevant stakeholders such as investigators, study team members, and study requestors. Moreover, key lessons learnt were collected from a total of 12 previously conducted NIS studies. Data were analysed thematically.

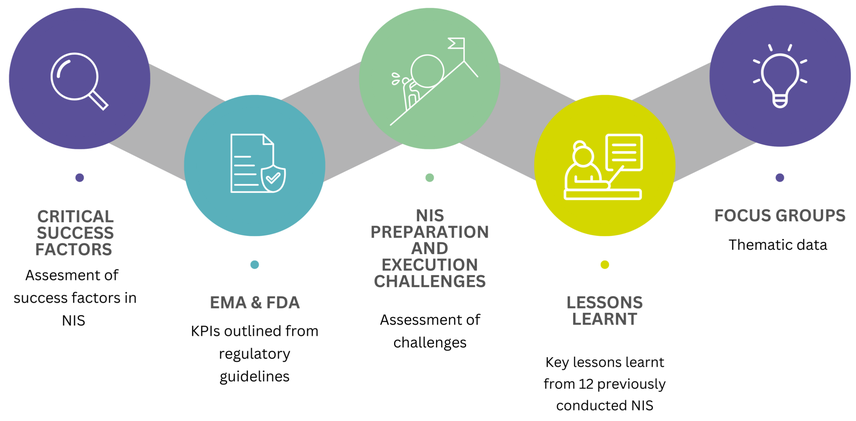



**Figure 1.** Study methodology

## RESULTS:

The analysis focused on mapping quality compliance requirements at different stages of multi-country NIS studies, such as study design, data collection, data management, ethical considerations, regulatory compliance, study team qualifications, study monitoring, safety reporting, collaboration, and communication Table 1). We have identified overall 9 critical success factors of NIS that could impact the quality of the NIS lifecycle.

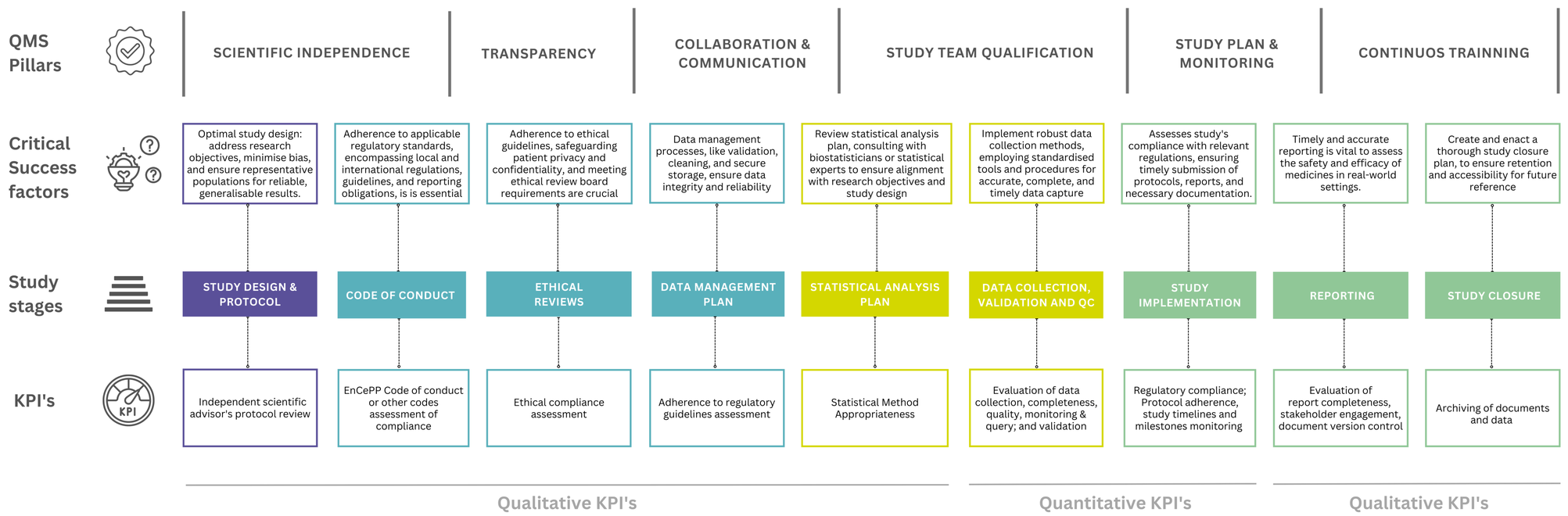
**Table 1.** Assessment of critical success factors within the current EMA and FDA guidelines

| | EMA | FDA |
|---|---|---|
| Study Design and Protocol Development | A well-defined study protocol should include study objectives, methodology, data collection procedures, patient selection criteria, and a statistical analysis plan. The study population should be well-defined and representative of the intended patient population. | The study design should be well-planned, scientifically rigorous, and capable of addressing the research objectives. The FDA encourages the use of appropriate comparators and control groups. |
| Data Collection, Validation and QC | Adequate measures should be taken to ensure high-quality data collection, including standardised data collection forms, appropriate training of investigators, and data validation procedures. | Adequate measures should be implemented to ensure accurate and reliable data collection, including appropriate data collection tools, standardised procedures, and quality control checks. |
| Ethical Considerations | NIS should comply with ethical principles, including patient privacy, informed consent, and appropriate use of data protection measures. | NIS should prioritise patient safety, including appropriate measures to minimise risks associated with data collection and analysis. |

| | EMA | FDA |
|---|---|---|
| Statistical Analysis | Statistical methods used for data analysis should be appropriate, well-documented, and transparent. EMA emphasises the importance of addressing confounding, bias, and potential sources of error. | The FDA expects rigorous and appropriate statistical methods to be employed, accounting for potential confounders and biases. The choice of statistical analyses should be justified and well-documented. |
| Reporting | Study results should be reported in a clear, comprehensive, and transparent manner, following the guidelines provided by the EMA. | The study results should be reported in a clear, concise, and transparent manner, following the FDA's guidelines for the presentation and interpretation of NIS data. |
| Regulatory Compliance | NIS should adhere to relevant regulatory requirements, including patient privacy and confidentiality, informed consent, and compliance with applicable laws and regulations. | |

We have identified overall 9 critical success factors of NIS that could impact the quality of the NIS lifecycle. Moreover, we developed more than 20 quantitative and qualitative KPIs covering different NIS stages. Some of the identified critical success factors and KPIs are original, while others have been adjusted from existing guidelines when its application needed enhancement.

**Figure 2.** NIS critical factors and KPIs for Quality Compliance Assessment




## CONCLUSIONS:

This study aims to improve the quality of multi-country NIS by using KPIs for compliance and monitoring. Implementing these KPIs will lead to higher standards and adherence to quality in post-marketing authorisation studies. Identifying critical success factors provides a framework for addressing key aspects of study quality, resulting in more robust and trustworthy NIS results that benefit patients and contribute to evidence-based healthcare decision-making.

## REFERENCES:

1. *EMA Guideline on Good Pharmacovigilance Practices (GVP) - Module VIII: Post-authorisation Safety Studies (Rev 3):*
2. *EMA Guideline on Good Pharmacovigilance Practices (GVP) - Module IX: Signal Management (Rev 2)*
3. *FDA Guidance for Industry: Postmarketing Studies and Clinical Trials—Implementation of Section 505(o)(3) of the Federal Food, Drug, and Cosmetic Act*
4. *FDA Guidance for Industry: Postmarketing Safety Reporting for Human Drug and Biological Products Including Vaccines*

***Conflicts of interest***

*Teamit experts conduct research for regulators and vaccine manufacturers according to the ENCePP code of conduct*