

COST PER PATIENT IN NON-INTERVENTIONAL STUDIES AND ADDED VALUE OF DIRECT TO PATIENT CONTACT SERVICE

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Objectives

- There are very limited data describing patient cost in Non-Interventional Studies (NIS) based on a prospective longitudinal patient follow-up design.
- In addition to study outcome concerns arising from patients lost to follow-up (LFU) in pharmacoepidemiology and pharmacovigilance studies, the financial impact of LFU can be significant.
- The objectives of the present analysis were:
 - To determine the cost per patient in prospective longitudinal NIS and to identify variables that may affect this patient cost;
 - To determine theoretical cost of patients lost to follow-up (LFU) and financial benefits that can be expected from LFU minimization through Direct to Patient Contact service (DPC).

Methods

- An analysis of Mapi Real-World Evidence (RWE) proposals and budgets submitted to sponsors in 2013 was performed.
- Criteria to select proposals and associated budgets included the presence of all following elements: non-interventional study, prospective and longitudinal patient follow-up, full Contract Research Organization (CRO) services. Criteria to exclude proposals/budgets included the presence of one of the following elements: retrospective study design or chart review, patient-reported outcome validation study, big data study, missing study design information, missing budget information, < 3 CRO services provided.
- Analyses were performed according to patient sample size, study duration, disease category, and different hypothesis for LFU rates.

Results

- Out of 96 proposals and budgets submitted in 2013, 20 met all the above inclusion criteria. Sponsor geographic origin was well balanced (11 European, 9 US), and most of the studies were international (10 global, 7 regional, 3 single country). See Table 1 for more details.

Table 1. General description of selected proposals (N = 20)

	Mean	Median	Minimum	Maximum
Study duration	5 years	2.1 years	0.5 year	15 years
Patient sample size	1,376	527	150	5,900
Study budget	6,127,269 €	4,006,260€	472,427 €	22,726,403 €

Figure 1. Mean annual cost (€) per patient according to study duration (N = 20)

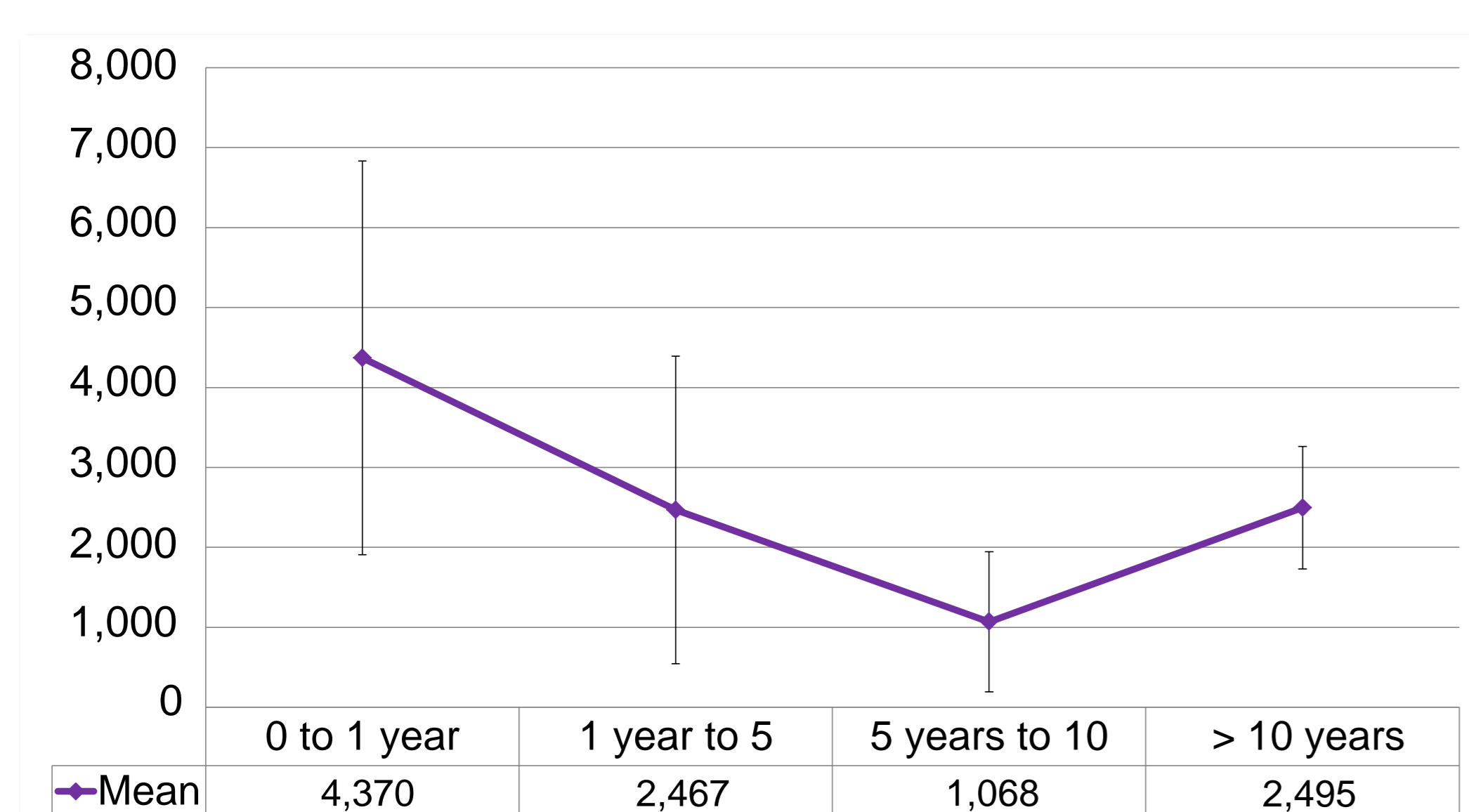


Figure 2. Mean annual cost (€) per patient according to disease category

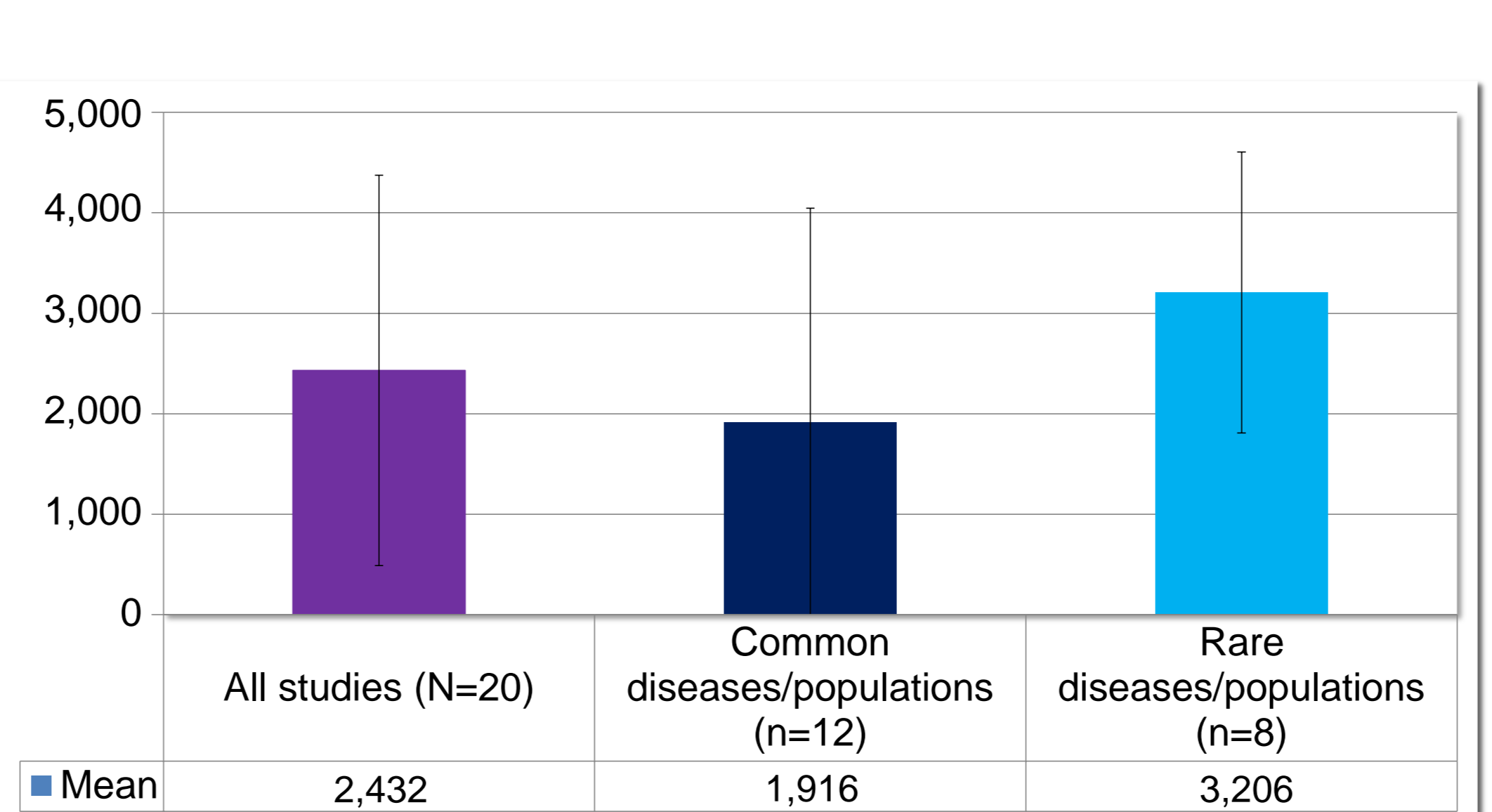
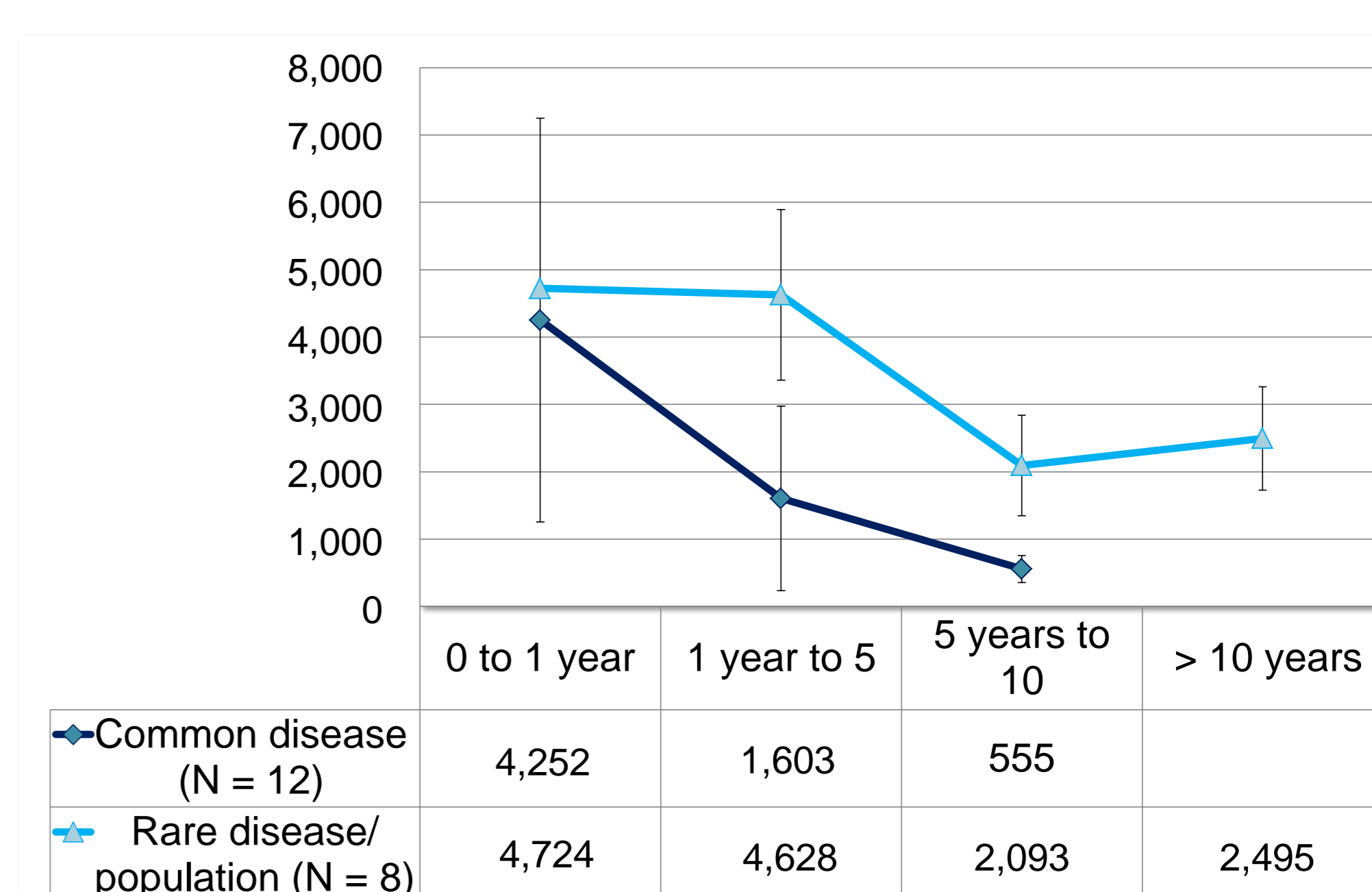


Figure 3. Comparison of the mean annual cost (€) per patient in common and rare diseases/populations



- Based on Table 2, additional theoretical tables were developed to provide guidance on cost saving amounts if DPC service is included in the study budget, or not, according to three variables and associated modalities:

- The *expected* patient LFU rate without DPC service, as based on sponsor's indication or Mapi's experience of the disease/population → modalities included LFU rates of 10% - 20% - 30% - 40% and 50%
- The *expected* patient LFU rate with DPC service, as based on DPC service metrics gathered over the last 13 years and more than 50 studies → modalities included LFU rates of 5% - 10%
- The *expected* patient sample size → modalities included 100; 500; 1,000; and 5,000 patients.
- The calculation to obtain the theoretical cost of LFU is:

Mean overall cost per patient x Expected sample size x Expected LFU rate

- The interpretation is: if the cost-saving amount is higher than an X estimated budget developed for a complete DPC service (which includes costs for the set-up, patient contact service management, and monthly coordination), it is *financially worthwhile* to add DPC service in the study design.

- Table 3 is an extract from the theoretical tables that provides cost saving amounts for all diseases (N = 20 proposals), and rare diseases (n= 8 proposals) for an expected sample size of 500 patients; these amounts are quite substantial, especially in rare diseases.

Table 2. Mean annual cost per patient vs. mean overall cost per patient according to mean study duration

Study duration (years)	Mean follow-up duration (years)	ALL DISEASES		COMMON DISEASES		RARE DISEASES	
		Mean annual cost/patient (€)	Mean overall cost/patient (€)	Mean annual cost/patient (€)	Mean overall cost/patient (€)	Mean annual cost/patient (€)	Mean overall cost/patient (€)
0 to 1	0.5	4,370	2,185	4,252	2,126	4,724	2,362
1 to 5	2.5	2,467	6,168	1,603	4,008	4,628	11,570
5 to 10	7.5	1,068	8,010	555	4,163	2,093	15,698
> 10	10	2,495	24,950	/	/	2,495	24,950

Table 3. Cost saving amounts for all diseases and rare diseases for a sample size of 500 patients

	All Diseases			Rare Diseases		
	Cost lost if no DPC	Cost lost if DPC	Cost saving	Cost lost if no DPC	Cost lost if DPC	Cost saving
Expected LFU Rate 10%			Δ			Δ
0 to 1 year	109,250	54,625	54,625	118,100	59,050	59,050
1 to 5 years	308,375	154,188	154,188	578,500	289,250	289,250
5 to 10 years	400,500	200,250	200,250	784,875	392,438	392,438
>10 years*	1,247,500	623,750	623,750	1,247,500	623,750	623,750
Expected LFU Rate 20%			Δ			Δ
0 to 1 year	218,500	54,625	163,875	236,200	59,050	177,150
1 to 5 years	616,700	154,188	462,563	1,157,000	289,250	867,750
5 to 10 years	801,000	200,250	600,750	1,569,750	392,438	1,177,313
>10 years*	2,495,000	623,750	1,871,250	2,495,000	623,750	1,871,250
Expected LFU Rate 30%			Δ			Δ
0 to 1 year	327,750	109,250	218,500	354,300	118,100	236,200
1 to 5 years	925,125	308,275	616,750	1,735,500	578,500	1,157,000
5 to 10 years	1,201,500	400,500	801,000	2,354,625	784,875	1,569,750
>10 years*	3,742,500	1,247,500	2,495,000	3,742,500	1,247,500	2,495,000
Expected LFU Rate 40%			Δ			Δ
0 to 1 year	437,000	109,250	327,750	472,400	118,100	354,300
1 to 5 years	1,233,500	308,275	925,125	2,314,000	578,500	1,735,500
5 to 10 years	1,602,000	400,500	1,201,500	3,139,500	784,875	2,354,625
>10 years*	4,990,000	1,247,500	3,742,500	4,990,000	1,247,500	3,742,500
Expected LFU Rate 50%			Δ			Δ
0 to 1 year	546,250	109,250	437,000	590,500	118,100	472,400
1 to 5 years	1,541,875	308,275	1,233,500	2,892,500	578,500	2,314,000
5 to 10 years	2,002,500	400,500	1,602,000	3,924,375	784,875	3,139,500
>10 years*	6,237,500	1,247,500	4,990,000	6,237,500	1,247,500	4,990,000

*For all diseases, patient costs and saving amounts are not representative for studies > 10 years as they only include rare diseases/populations study proposals.

Conclusion

Return on investment plays an important role for sponsors to determine if DPC strategies are valuable in a study. The financial investment may be beneficial regardless of the cost to ensure completion of the patients, thus meeting the scientific study objectives. And ultimately could generate cost savings as well.