Burden of illness & incidence/prevalence studies

In-depth epidemiology analysis to support the case for FDA Orphan designation

CLIENT NEED

US biotech Executive team sought insight and analysis on target rare diseases for their early asset portfolio, where no estimates of disease burden had been published.

OUR APPROACH

- Identified key diseases/sub populations with client
- Conducted data search to uncover all relevant information according to checklist criteria
- Performed systematic literature review with meta-analysis
- Presented natural history, treatment, signs and symptoms, diagnosis, life-expectancy and excess mortality
- Defined and built a comprehensive custom disease model

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OUTCOMES & VALUE

- Completed forensic analysis on expected course, severity and symptoms, and causes of death to support FDA negotiations
- Developed custom disease model to generate prevalent disease estimates, by country, age and gender along with genetic sub-types
- Delivered patient estimates to provide a robust, alternative perspective to Client's own view on disease severity and prevalence

SAMPLE DELIVERABLES

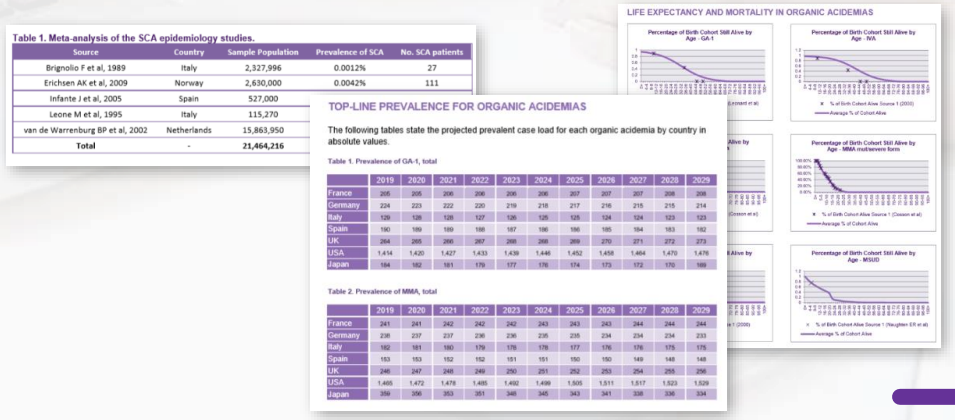

Table 1. Meta-analysis of the SCA epidemiology studies.

Source	Country	Sample Population	Prevalence of SCA	No. SCA patients
Brignolio F et al, 1989	Italy	2,327,996	0.0012%	27
Erichsen AK et al, 2009	Norway	2,630,000	0.0042%	111
Infante J et al, 2005	Spain	527,000		
Leone M et al, 1995	Italy	115,270		
van de Warrenburg BP et al, 2002	Netherlands	15,863,950		
Total	-	21,464,216		

LIFE EXPECTANCY AND MORTALITY IN ORGANIC ACIDEMIAS

TOP-LINE PREVALENCE FOR ORGANIC ACIDEMIAS

The following tables state the projected prevalent case load for each organic acidemia by country in absolute values.

Table 1. Prevalence of GA-1, total

	2019	2020	2021	2022	2023	2024	2025	2026	2027	2028	2029
France	205	205	206	206	206	206	207	207	207	208	208
Germany	224	223	222	220	219	218	217	216	215	215	214
Italy	129	128	128	127	126	125	125	124	124	123	123
Spain	190	189	189	188	187	186	186	185	184	183	182
UK	264	265	266	267	268	268	269	270	271	272	273
USA	1,414	1,420	1,427	1,433	1,439	1,446	1,452	1,458	1,464	1,470	1,476
Japan	184	182	181	179	177	176	174	173	172	170	169

Table 2. Prevalence of MMA, total

	2019	2020	2021	2022	2023	2024	2025	2026	2027	2028	2029
France	241	241	242	242	242	243	243	243	244	244	244
Germany	238	237	237	236	236	235	235	234	234	234	233
Italy	182	181	180	179	178	178	177	176	176	175	175
Spain	153	153	152	152	151	151	150	150	149	148	148
UK	246	247	248	249	250	251	252	253	254	255	256
USA	1,465	1,472	1,478	1,485	1,492	1,499	1,505	1,511	1,517	1,523	1,529
Japan	359	356	353	351	348	345	343	341	338	336	334

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