Appendix 2: Individual Outcomes of Therapy

Appendix 2 contains tabular summaries for those patients who achieved an objective MTT response (by study) and those who achieved Patient Benefit. Also included in this appendix are synopses for six patients. These are included to provide individual examples of the types of patients that were treated in the Phase III studies. For three of the patients, photographs of the patients prior to and after treatment are included.

1. Outcomes Tables

The following tables include individual outcomes for patients with objective response of the MTT or who achieved Patient Benefit in the blinded phase of studies 414 and 514:

Appendix 2: Individual Outcomes of Therapy

Table A2-1: Patients Who Achieved an MTT Response, Study 414 – Blinded Phase

Pt. No.	Treatment Group	Response	Duration of Response (days)	Time to Response		Site of 1° Cancer	MTT Location	Baseline MTT Vol. (cm^3)	Prior Chemotherapy	Previous RT for MTT or MTT Field?
				Days	# of Rxs					
5301	CDDP/epi gel	CR	168+	21	2	Nasal cavity	Nasopharyngeal	3.23	No	Yes
1995	CDDP/epi gel	CR	120+	14	2	Nasal cavity	Facial	0.500	Yes (Pt)	Yes
5111	CDDP/epi gel	CR	105+	28	3	Unknown	Facial	2.00	No	Yes
1998	CDDP/epi gel	CR	103+	14	2	Larynx	Cervical	3.00	Yes (Pt)	Yes
4964	CDDP/epi gel	CR	99+	47	5	Oral cavity	Cervical	8.75	Yes (Pt)	Yes
2000	CDDP/epi gel	CR	97+	28	2	Oropharynx	Cervical	0.625	No	Yes
5036	CDDP/epi gel	CR	95+	17	2	Oral cavity	Facial	7.81	Yes (Pt)	Yes
5348	CDDP/epi gel	CR	85+	21	3	Oral cavity	Cervical	13.5	Yes (Pt)	Yes
1993	CDDP/epi gel	CR	78+	35	5	Larynx	Cervical	2.50	No	Yes
2136	CDDP/epi gel	CR	77+	7	1	Salivary glands	Cervical	0.600	Yes (Pt)	Yes
5303	CDDP/epi gel	CR	64+	14	2	Oral cavity	Facial	0.900	Yes (Pt)	Yes
5134	CDDP/epi gel	CR	37+	9	1	Larynx	Cervical	1.25	No	Yes
5151	CDDP/epi gel	CR	36+	7	1	Oral cavity	Facial	17.5	No	Yes
1825	CDDP/epi gel	CR	34[a]	20	2	Unknown	Oral	0.504	No	No

Time to response: Time to beginning of PR or CR
Duration of response: calculated from the onset of PR or CR until "censored" at the time of relapse, initiation of confounding therapy, or death
"Pt" indicates platinum-based chemotherapy (cisplatin, carboplatin)
Plus sign (+) indicates that the tumor was responding at the time of the last evaluation
[a] Time to progression: 216+ days

Table A2-1: Patients Who Achieved an MTT Response, Study 414 – Blinded Phase (continued)

Pt. No.	Treatment Group	Response	Duration of Response (days)	Time to Response		Site of 1° Cancer	MTT Location	Baseline MTT Vol. (cm^3)	Prior Chemotherapy	Previous RT for MTT or MTT Field?
				Days	# of Rxs					
1797	CDDP/epi gel	PR	124+	21	2	Unknown	Cranial	6.00	Yes (Pt)	Yes
5372	CDDP/epi gel	PR	119+	35	5	Unknown	Facial	18.8	Yes (Pt)	Yes
5133	CDDP/epi gel	PR	99+	16	2	Oropharynx	Oral	4.00	No	Yes
5110	CDDP/epi gel	PR	75+	14	2	Oropharynx	Cervical	2.00	Yes (Pt)	Yes
1772	CDDP/epi gel	PR	53+	50	6	Oropharynx	Oral	11.3	Yes	Yes
2110	CDDP/epi gel	PR	43+	14	2	Oropharynx	Oral	7.50	Yes (Pt)	Yes
1992	CDDP/epi gel	PR	35[b]	14	2	Unknown	Cervical	2.00	Yes (Pt)	Yes

Time to response: Time to beginning of PR or CR
Duration of response: calculated from the onset of PR or CR until "censored" at the time of relapse, initiation of confounding therapy, or death
"Pt" indicates platinum-based chemotherapy (cisplatin, carboplatin)
Plus sign (+) indicates that the tumor was responding at the time of the last evaluation
[b] Time to progression: 49 days

Table A2-2: Patients Who Achieved an MTT Response, Study 514 – Blinded Phase

Pt. No.	Treatment Group	Response	Duration of Response (days)	Time to Response		Site of 1° Cancer	MTT Location	Baseline MTT Vol. (cm^3)	Prior Chemotherapy	Previous RT for MTT or MTT Field?
				Days	# of Rxs					
2732	CDDP/epi gel	CR	554+	10	1	Unknown	Oral	9	No	Yes
2681	CDDP/epi gel	CR	391+	14	2	Oral cavity	Facial	12.4	No	Yes
5565	CDDP/epi gel	CR	136+	60	6	Hypopharynx	Oral	0.75	No	No
2736	CDDP/epi gel	CR	113+	97	6	Oral cavity	Oral	4.0	Yes (Pt)	Yes
2686	CDDP/epi gel	CR	64+	28	3	Nasopharynx	Oral	2	No	Yes
2735	CDDP/epi gel	CR	57+	112	6	Oropharynx	Oral c	2.92	No	Yes
5854	CDDP/epi gel	CR	55+	14	2	Oral cavity	Cervical[a]	2.52	No	No
5495	Placebo	CR	54+	56	5	Unknown	Cervical	0.75	No	Yes
2687	CDDP/epi gel	CR	49+	105	6	Oropharynx	Oral	1	No	Yes
2731	CDDP/epi gel	CR	46+	46	5	Unknown	Facial	10.9	No	Yes
5496	CDDP/epi gel	PR	93+	35	4	Oral cavity	Oral	1.25	No	Yes
2688	CDDP/epi gel	PR	84+	63	6	Oropharynx	Oral	1.5	Yes (Pt)	Yes
2753	CDDP/epi gel	PR	64+	104	6	Larynx	Oral	15.8	No	Yes
2541	CDDP/epi gel	PR	58+	162	1	Larynx	Cervical[b]	4.95	No	Yes
5494	CDDP/epi gel	PR	30+	21	3	Oral cavity	Oral	3.75	Yes[c]	Yes

Time to response: Time to beginning of PR or CR
Duration of response: calculated from the onset of PR or CR until "censored" at the time of relapse, initiation of confounding therapy, or death
"Pt" indicates platinum-based chemotherapy (cisplatin, carboplatin)
Plus sign (+) indicates that the tumor was responding at the time of the last evaluation
[a]Upper anterior chest wall
[b]Supraclavicular
[c] "LV," 5-FU

Table A2-3: Treatment Goals for Patients Who Achieved an MTT Response, Study 414 – Blinded Phase

Pt. No.	Treatment Group	Response	Patient Benefit Achieved	MTT Location	1° Treatment Goal (Patient)		1° Treatment Goal (Investigator)	
					Goal	Outcome	Goal	Outcome
5301	CDDP/epi gel	CR	Yes	Nasopharyngeal	Wound care	Same	Prevention of invasion	Met
1998	CDDP/epi gel	CR	Yes	Cervical	Pain control	Same	Prevention of invasion	Met
5134	CDDP/epi gel	CR	Yes	Cervical	Pain control	Met	Prevention of tumors breaking the skin	Same
1993	CDDP/epi gel	CR	Yes	Cervical	Physical appearance	Worse[a]	Prevention of invasion	Met
2136	CDDP/epi gel	CR	Yes	Cervical	Physical appearance	Same	Prevention of tumors breaking the skin	Met
1825	CDDP/epi gel	CR	Yes	Oral	Obstructive symptom	Same	Pain control	Met
5036	CDDP/epi gel	CR	Yes	Facial	No goal selected	n/a	Prevention of tumors breaking the skin	Met
1995	CDDP/epi gel	CR	Yes	Facial	No goal selected	n/a	Prevention of invasion	Met
5303	CDDP/epi gel	CR	Yes	Facial	No goal selected	n/a	Prevention of tumors breaking the skin	Met
5111	CDDP/epi gel	CR	No	Facial	Wound care	Same	Wound care	Same
5348	CDDP/epi gel	CR	No	Cervical	Wound care	Same	Wound care	Same
4964	CDDP/epi gel	CR	No	Cervical	Obstructive symptom	Same	Obstructive symptom	Same
2000	CDDP/epi gel	CR	No	Cervical	Pain control	Same	Prevention of tumors breaking the skin	Unmet
5151	CDDP/epi gel	CR	No	Facial	No goal selected	n/a	Obstructive symptom	Same
1797	CDDP/epi gel	PR	Yes	Cranial	No goal selected	n/a	Prevention of tumors breaking the skin	Met
5372	CDDP/epi gel	PR	No	Facial	Physical appearance	Same	Obstructive symptom	Same
5133	CDDP/epi gel	PR	No	Oral	Obstructive symptom	Same	Obstructive symptom	Same
5110	CDDP/epi gel	PR	No	Cervical	Obstructive symptom	Same	Obstructive symptom	Same
1772	CDDP/epi gel	PR	No	Oral	Pain control	Worse	Pain control	Worse
2110	CDDP/epi gel	PR	No	Oral	Pain control	Same	Obstructive symptom	Worse
1992	CDDP/epi gel	PR	No	Cervical	Ability to smell	Worse	Prevention of invasion	Met

[a]The goal did not fail for ≥28 days, thereby meeting the outcome criteria for “same”

Table A2-4: Treatment Goals for Patients Who Achieved an MTT Response, Study 514 – Blinded Phase

Pt. No.	Treatment Group	MTT Response Status	Patient Benefit Achieved?	MTT Location	1° Treatment Goal — Patient		1° Treatment Goal — Investigator	
					Goal	Outcome	Goal	Outcome
2736	CDDP/epi gel	CR	Yes	Oral	Wound care	Met	Wound care	Met
2735	CDDP/epi gel	CR	Yes	Oral	Wound care	Met	Wound care	Met
5495	Placebo	CR	Yes	Cervical	Physical appearance	Met	Physical appearance	Met
2687	CDDP/epi gel	CR	Yes	Oral	Obstructive symptom	Met	Obstructive symptom	Met
2732	CDDP/epi gel	CR	Yes	Oral	No goal selected	—	Pain control	Met
5854	CDDP/epi gel	CR	Yes	Cervical	No goal selected	—	Prevention of tumors breaking skin	Met
2681	CDDP/epi gel	CR	No	Facial	Physical appearance	Same	Physical appearance	Same
5565	CDDP/epi gel	CR	No	Oral	Obstructive symptom	Same	Obstructive symptom	Same
2686	CDDP/epi gel	CR	No	Oral	Obstructive symptom	Not Met	Obstructive symptom	Not Met
2731	CDDP/epi gel	CR	No	Facial	Pain control	Same	Pain control	Same
2688	CDDP/epi gel	PR	Yes	Oral	Obstructive symptom	Met	Obstructive symptom	Met
5496	CDDP/epi gel	PR	No	Oral	Obstructive symptom	Same	Obstructive symptom	Not met
2753	CDDP/epi gel	PR	No	Oral	Pain control	Not Met	Obstructive symptom	Not Met
2541	CDDP/epi gel	PR	No	Cervical	Physical appearance	Met	Prevention of tumors breaking skin	Not Met
5494	CDDP/epi gel	PR	No	Oral	Pain control	Same	Pain control	Same

Table A2-5: Patients Who Achieved Patient Benefit, Study 414 – Blinded Phase

Pt. No.	Treatment Group	MTT Response Status	Stra	MTT Location	1° Treatment Goal — Patient		1° Treatment Goal — Investigator	
					Goal	Outcome	Goal	Outcome
1998	CDDP/epi gel	CR	1	Cervical	Pain control	Same	Prevention of invasion	Met
5134	CDDP/epi gel	CR	1	Cervical	Pain control	Met	Prevention of tumors breaking the skin	Same
1825	CDDP/epi gel	CR	1	Oral	Obstructive symptom	Same	Pain control	Met
1993	CDDP/epi gel	CR	1	Cervical	Physical appearance	Worse[a]	Prevention of invasion	Met
2136	CDDP/epi gel	CR	1	Cervical	Physical appearance	Same	Prevention of tumors breaking the skin	Met
5301	CDDP/epi gel	CR	1	Nasopharyngeal	Wound care	Same	Prevention of invasion	Met
1995	CDDP/epi gel	CR	1	Facial	No goal selected	n/a	Prevention of invasion	Met
5303	CDDP/epi gel	CR	1	Facial	No goal selected	n/a	Prevention of tumors breaking the skin	Met
5036	CDDP/epi gel	CR	2	Facial	No goal selected	n/a	Prevention of tumors breaking the skin	Met
1797	CDDP/epi gel	PR	2	Cranial	No goal selected	n/a	Prevention of tumors breaking the skin	Met
2064	CDDP/epi gel	SD	1	Nasopharyngeal	Ability to hear	Same	Prevention of invasion	Met
1996	CDDP/epi gel	SD	1	Cervical	No goal selected	n/a	Prevention of invasion	Met
5253	CDDP/epi gel	SD	1	Cervical	No goal selected	n/a	Prevention of tumors breaking the skin	Met
5278	CDDP/epi gel	SD	1	Cervical	No goal selected	n/a	Prevent obstruction	Met
5277	CDDP/epi gel	SD	1	Facial	Pain control	Same	Prevention of tumors breaking the skin	Met
1871	CDDP/epi gel	SD	2	Cervical	Obstructive symptom	Same	Prevention of invasion	Met
2183	CDDP/epi gel	SD	2	Cervical	Pain control	Same	Prevent obstruction	Met
2159	CDDP/epi gel	SD	2	Oral	Pain control	Met	Pain control	Same
1944	CDDP/epi gel	PD	1	Laryngopharyngeal	Pain control	Same	Prevent obstruction	Met
5302	CDDP/epi gel	PD	1	Nasopharyngeal	Ability to see	Same	Prevention of tumors breaking the skin	Met
2157	CDDP/epi gel	PD	2	Facial	No goal selected	n/a	Prevent obstruction	Met
1795	CDDP/epi gel	PD	2	Cervical	No goal selected	n/a	Prevent obstruction	Met
2015	CDDP/epi gel	NER	1	Cervical	No goal selected	n/a	Prevention of invasion	Met
5396	CDDP/epi gel	NER	2	Nasopharyngeal	Obstructive symptom	Same	Prevention of invasion	Met
2014	CDDP/epi gel	NER	2	Facial	No goal selected	n/a	Prevention of tumors breaking the skin	Met

[a] The goal did not fail for ≥28 days, thereby meeting the outcome criteria for “same”

Table A2-6: Patients Who Achieved Patient Benefit, Study 514 – Blinded Phase

Pt. No.	Treatment Group	MTT Response Status	Stratum	MTT Location	1° Treatment Goal — Patient		1° Treatment Goal — Investigator	
					Goal	Outcome	Goal	Outcome
2687	CDDP/epi gel	CR	1	Oral	Obstructive symptom	Met	Obstructive symptom	Met
2735	CDDP/epi gel	CR	1	Oral	Wound care	Met	Wound care	Met
2736	CDDP/epi gel	CR	1	Oral	Wound care	Met	Wound care	Met
5495	CDDP/epi gel	CR	1	Cervical	Physical appearance	Met	Physical appearance	Met
5854	CDDP/epi gel	CR	1	Cervical	No goal selected	—	Prevention of tumors breaking skin	Met
2732	CDDP/epi gel	CR	2	Oral	No goal selected	—	Pain control	Met
2688	CDDP/epi gel	PR	1	Oral	Obstructive symptom	Met	Obstructive symptom	Met
2253	Placebo	SD	1	Cervical	No goal selected	—	Prevention of tumors breaking skin	Met
2366	CDDP/epi gel	SD	2	Facial	Wound care	Met	Wound care	Met
2564	CDDP/epi gel	SD	2	Cervical	Pain control	Met	Pain control	Met
2443	Placebo	SD	2	Cervical	Obstructive symptom	Same	Prevent obstruction	Met
2421	CDDP/epi gel	PD	1	Cervical	Pain control	Met	Pain control	Same
5421	CDDP/epi gel	PD	1	Cervical	Pain control	Met	Pain control	Same
5588	CDDP/epi gel	PD	2	Cervical	Wound care	Same	Prevention of tumors breaking skin	Met

Appendix 2: Individual Outcomes of Therapy

2. Patient Synopses

CDDP/epi gel is a novel cancer treatment modality designed to achieve local tumor control. Following are patient profiles and photographs that illustrate the type of patients treated, the extent of previous treatment, the course of resolution of tumors, including local tissue conditions, the quality of response and the associated clinical benefits. Four of the following six patients were recorded as having both Patient Benefit and objective tumor response. Two of the patients (2137 and 5348) had evidence of other clinical benefits based upon the risks of continued tumor growth in the threatening location of these tumors or other secondary or unexpected benefits recorded. Two of these patients (2137 and 2660) illustrate the rapid tumor progression that was seen during the placebo blinded phase, yet was followed by response to open-label CDDP/epi gel. All of these patients have complex medical histories. Synopses are provided for the following patients:

Patient	Study	Randomized Treatment Group
2137*	414	Placebo
2660*	514	Placebo
5348*	414	CDDP/epi gel
1998	514	CDDP/epi gel
5134	414	CDDP/epi gel
5301	414	CDDP/epi gel

*Photographs of these patients included